

## Appendix S1

### Results of searches on clinicaltrials.gov – 17 September 2021

All results from clinicaltrials.gov were completely assessed for eligibility after removal of duplicates.

| Other terms | Condition/disease  |
|-------------|--|
| Vismodegib  | basal cell nevus syndrome → 7 results                    |
| Vismodegib  | multiple basal cell carcinomas → 7 results, 7 duplicates |
| Vismodegib  | basal cell carcinoma → 28 results, 6 duplicates          |
| GDC-0449    | basal cell nevus syndrome → 6 results, 6 duplicates      |
| GDC-0449    | multiple basal cell carcinomas → 6 results, 6 duplicates |
| GDC-0449    | basal cell carcinoma → 23 results, 23 duplicates         |

n = 29 unique results, n=2 inclusions

| Study  | Inclusion or exclusion | Reason  |
|--|------------------------|---|
| Nivolumab With Vismodegib in Patients With Basal Cell Nevus Syndrome   | Exclusion              | <ul style="list-style-type: none"> <li>- Study was withdrawn</li> <li>- Combination therapy</li> </ul>  |
| Trial Comparing the Effects of Intermittent Vismodegib vs. PDT in Patients With Multiple Basal Cell Carcinomas | Exclusion              | <ul style="list-style-type: none"> <li>- Completion in 2015 but no available results</li> </ul>   |
| To Determine The Efficacy and Safety of GDC-0449 in Patients With Basal Cell Nevus Syndrome (BCNS)             | Inclusion              | Randomized controlled trial comparing vismodegib 150mg daily to placebo in patients with BCNS   |
| Photodynamic Therapy and Vismodegib for Multiple Basal Cell Carcinomas   | Exclusion              | <ul style="list-style-type: none"> <li>- Completion in 2017 but no results available</li> <li>- Combination therapy</li> </ul>                                |
| A Study of Two Vismodegib Regimens in Participants With Multiple Basal Cell Carcinomas                         | Inclusion              | Randomized controlled trial comparing two vismodegib dosing regimens in patients with BCNS or multiple BCCs.  |
| <a href="#">Safety and Efficacy of ASN-002 Combined With a Hedgehog Pathway Inhibitor</a>                      | Exclusion              | <ul style="list-style-type: none"> <li>- Still recruiting</li> <li>- Combination therapy</li> </ul>   |
| <a href="#">Levocarnitine in Treating Patients With Vismodegib-Associated Muscle Spasms</a>                    | Exclusion              | <ul style="list-style-type: none"> <li>- Completion in 2017 but no results available</li> <li>- Does not provide answers to our research questions</li> </ul> |
| <a href="#">Vismodegib for Treatment of Basal Cell Carcinoma</a>   | Exclusion              | <ul style="list-style-type: none"> <li>- Completion in 2013, only 3 patients enrolled, no results</li> <li>- Vismodegib as neoadjuvant therapy</li> </ul>     |
| <a href="#">Neoadjuvant Vismodegib in Patients With Large and/or Recurrent Resectable Basal Cell Carcinoma</a> | Exclusion              | <ul style="list-style-type: none"> <li>- Study status unknown</li> <li>- Vismodegib as neoadjuvant therapy</li> </ul>   |

|   |           |   |
|---|-----------|---|
| <a href="#"><u>Study Evaluating the Interest of <b>Vismodegib</b> as Neo-adjuvant Treatment of <b>Basal Cell Carcinoma</b> (BCC)</u></a>  | Exclusion | <ul style="list-style-type: none"> <li>- Vismodegib as neoadjuvant therapy</li> <li>*results available</li> </ul>   |
| <a href="#"><u>A Study Evaluating the Efficacy and Safety of <b>Vismodegib</b> (GDC-0449, Hedgehog Pathway Inhibitor) in Patients With Advanced <b>Basal Cell Carcinoma</b></u></a> | Exclusion | <ul style="list-style-type: none"> <li>- Outcomes focused on response of advanced basal cell carcinoma</li> <li>*Results available, ERIVANCE trial</li> </ul>   |
| <a href="#"><u>A Study Evaluating the Efficacy and Safety of <b>Vismodegib</b> (GDC-0449) in Operable <b>Basal Cell Carcinoma</b></u></a>   | Exclusion | <ul style="list-style-type: none"> <li>- Outcomes focused on efficacy of vismodegib on one, new, operable, nodular BCC</li> <li>- BCNS patients were excluded</li> <li>*Results available</li> </ul>      |
| <a href="#"><u>A Study to Assess the Effectiveness and Safety of <b>Vismodegib</b> (Erivedge®) in Participants With Advanced <b>Basal Cell Carcinoma</b> (BCC)</u></a>              | Exclusion | <ul style="list-style-type: none"> <li>- Completed in 2020, no results</li> <li>- Outcomes focused on response of advanced basal cell carcinoma but did include BCNS patients</li> </ul>                  |
| <a href="#"><u>A Study of <b>Vismodegib</b> With Surgery in Participants With Previously Untreated <b>Basal Cell Carcinoma</b></u></a>  | Exclusion | <ul style="list-style-type: none"> <li>- Outcomes focused on change in BCC and surgical defect area at Mohs micrographic surgery after treatment of one BCC</li> <li>*Results available</li> </ul>        |
| <a href="#"><u><b>Vismodegib</b> on Locally Advanced <b>Basal Cell Carcinoma</b> Under Real World Conditions</u></a>  | Exclusion | <ul style="list-style-type: none"> <li>- Study focuses on prospective data of vismodegib for laBCC (inappropriate for surgery or radiotherapy) in Germany</li> <li>*Results available</li> </ul>          |
| <a href="#"><u>STEVIE: A Study of <b>Vismodegib</b> in Patients With Locally Advanced or Metastatic <b>Basal Cell Carcinoma</b></u></a>   | Exclusion | <ul style="list-style-type: none"> <li>- Study focuses on outcomes laBCC and mBCC.</li> <li>*Results available, STEVIE trial</li> </ul>   |
| <a href="#"><u><b>Vismodegib</b> in <b>Basal Cell Carcinomas</b> (BCC) Chemoprevention</u></a>  | Exclusion | <ul style="list-style-type: none"> <li>- Study focuses on vismodegib as chemoprevention for development of new BCCs in high risk subjects</li> <li>- Study was terminated due to low enrolment</li> </ul> |
| <a href="#"><u><b>Pembrolizumab</b> With or Without <b>Vismodegib</b> in Treating Metastatic or Unresectable <b>Basal Cell Skin Cancer</b></u></a>                                  | Exclusion | <ul style="list-style-type: none"> <li>- Combination therapy for aBCC</li> <li>- Compared pembrolizumab to pembrolizumab+vismodegib</li> <li>*Results available</li> </ul>                                |
| <a href="#"><u><b>Vismodegib</b> in Treating Patients With <b>Basal Cell Carcinoma</b> (BCC)</u></a>  | Exclusion | <ul style="list-style-type: none"> <li>- Outcomes focused on change in BCC and surgical defect area at Mohs micrographic surgery after treatment of one BCC</li> <li>*Results available</li> </ul>        |
| <a href="#"><u>Study Evaluating the Efficacy of Oral <b>Vismodegib</b> in Various Histologic Subtypes</u></a>   | Exclusion | <ul style="list-style-type: none"> <li>- Included maximum of 4 BCCs per patient</li> <li>- Outcomes focuses on efficacy of different histological</li> </ul>  |

|   |           |   |
|---|-----------|---|
|   |           | subtypes<br>*Results available  |
| <a href="#">Relationship Between Pharmacokinetics and Safety of <b>Vismodegib</b> - OPTIVISMO-1</a>   | Exclusion | - Still recruiting<br>- Outcomes focuses on relationship between plasma concentration of vismodegib and occurrence of adverse effects   |
| <a href="#">Phase II Study of Radiation Therapy and <b>Vismodegib</b> for Advanced Head/Neck <b>Basal Cell Carcinoma</b></a>  | Exclusion | - Combination therapy for laBCC of the head and neck, BCNS patients are excluded<br>*Results available  |
| <a href="#">To Assess The Efficacy And Safety Of <b>Vismodegib</b> And Radiotherapy In Advanced <b>Basal Cell Carcinoma</b></a>   | Exclusion | - Terminated due to low recruitment rate<br>- Combination therapy for patients with high risk of relapse BCC in technically difficult areas for surgery, BCNS patients are excluded |
| <a href="#">VISmodegib for ORbital and Periocular <b>Basal Cell Carcinoma</b></a>   | Exclusion | - Outcome focuses on efficacy of one orbital/periocular aBCC after vismodegib and with or without surgery<br>*results available   |
| <a href="#">Observational Study to Determine the Effectiveness and Safety of <b>Vismodegib</b> (<b>Erivedge</b>®) in Participants With Locally Advanced <b>Basal Cell Carcinoma</b> (laBCC)</a>                   | Exclusion | - No results available<br>*Study focuses on prospective data of vismodegib for laBCC (inappropriate for surgery or radiotherapy) in Germany   |
| <a href="#">Observational Study of <b>Vismodegib</b> (<b>Erivedge</b>) in Patients Treated in Argentina</a>   | Exclusion | - Study focuses on prospective data of vismodegib for aBCC (inappropriate for surgery or radiotherapy) in Argentina<br>*Results available   |
| <a href="#">A Study of <b>Vismodegib</b> (<b>GDC-0449</b>) in Patients Treated With <b>Vismodegib</b> in a Previous Genentech-sponsored Phase I or II Cancer Study</a>  | Exclusion | - Outcomes focuses on adverse events in patients treated with vismodegib regardless for what tumour<br>*Results available   |
| <a href="#">Pilot Biomarker Trial to Evaluate the Efficacy of Itraconazole in Patients w/ <b>Basal Cell Carcinomas</b></a>  | Exclusion | - Outcomes focuses on tumor biomarkers in patients with one BCC >4mm diameter that would get surgically removed   |
| <a href="#">A Study of Hedgehog Pathway Inhibitor <b>GDC-0449</b> in Patients With Locally Advanced or Metastatic Solid Tumors That Are Refractory to Standard Therapy or for Whom No Standard Therapy Exists</a> | Exclusion | - Outcomes focuses on plasma concentrations of vismodegib in patients treated with vismodegib regardless for what tumour<br>*Results available                                      |

BCNS = basal cell nevus syndrome BCC = basal cell carcinoma laBCC = locally advanced basal cell carcinoma aBCC = advanced basal cell carcinoma

**Other terms**

Sonidegib

Sonidegib

Sonidegib

LDE225

LDE225

LDE225

**Condition/disease**

basal cell nevus syndrome → 4 results

multiple basal cell carcinomas → 6 results, 4 duplicates

basal cell carcinoma → 14 results, 7 duplicates

basal cell nevus syndrome → 4 results, 4 duplicates

multiple basal cell carcinomas → 6 results, 6 duplicates

basal cell carcinoma → 14 results, 14 duplicates

**n=13 unique results, n=2 inclusions**

| Study  | Inclusion or exclusion | Reason  |
|--|------------------------|---|
| <a href="#">A Trial to Evaluate the Safety, Local Tolerability, Pharmacokinetics and Pharmacodynamics of LDE225 on Skin Basal Cell Carcinomas in Gorlin Syndrome Patients</a>        | Inclusion              | Randomized vehicle-controlled trial to evaluate safety, tolerability, pharmacokinetics- and dynamics of topical LDE225 in patients with multiple BCCs and BCNS  |
| <a href="#">Efficacy, Safety and Pharmacokinetics of Oral LDE225 in Treatment of Patients With Nevoid Basal Cell Carcinoma Syndrome (NBCCS)</a>                                      | Inclusion              | Randomized proof-of-concept dose-ranging trial to evaluate efficacy, safety and pharmacokinetics of oral LDE225 in patients with BCNS and multiple basal cell carcinomas.   |
| <a href="#">Efficacy, Safety and Tolerability of Topically Applied LDE225 Cream (Hedgehog Pathway Inhibitor) in Adult Patients With Nevoid Basal Cell Carcinoma Syndrome (NBCCS)</a> | Exclusion              | <ul style="list-style-type: none"> <li>- Withdrawn before participants were enrolled</li> <li>- Randomized vehicle-controlled trial of topical LDE225 in patients with multiple BCCs and BCNS</li> </ul>  |
| <a href="#">Pilot Study of Sonidegib and Buparlisib in Treating Patients With Advanced or Metastatic Basal Cell Carcinoma</a>  | Exclusion              | <ul style="list-style-type: none"> <li>- Combination therapy for patients with laBCC or mBCC</li> <li>- Terminated (business decision) with 10 enrollments</li> </ul> *Results available  |
| <a href="#">To Evaluate the Safety, Local Tolerability, PK and PD of LDE225 on Sporadic Superficial and Nodular Skin Basal Cell Carcinomas (sBCC)</a>                                | Exclusion              | <ul style="list-style-type: none"> <li>- Randomized vehicle-controlled trial of topical LDE225 in one sporadic superficial or nodular BCC</li> <li>- Terminated due to insufficient efficacy in superficial BCC with the formulation and treatment conditions, results available</li> </ul> |
| <a href="#">Post-authorization Safety Study on the Long Term Safety of Sonidegib in Patients With Locally Advanced Cell Carcinoma</a>  | Exclusion              | <ul style="list-style-type: none"> <li>- Still recruiting</li> <li>- Observational study to assess safety of sonidegib for laBCC</li> </ul>   |
| <a href="#">A Phase II Study of Efficacy and Safety in Patients With Locally Advanced or Metastatic Basal Cell Carcinoma</a>   | Exclusion              | <ul style="list-style-type: none"> <li>- Results focuses on outcomes for laBCC and mBCC patients</li> </ul> *Results available, BOLT study  |

|  |           |  |
|--|-----------|--|
| <a href="#">Pilot <b>LDE225</b> in Locally Advanced or Metastatic BCC + Previously Tx Non-LDE225 Smoothened Inhibitors</a>                         | Exclusion | <ul style="list-style-type: none"> <li>- Results focuses on outcomes for laBCC and mBCC patients</li> <li>*Results available</li> </ul>  |
| <a href="#">Tailored <b>Sonidegib</b> Schedule After Complete Response in BCC</a>  | Exclusion | <ul style="list-style-type: none"> <li>- Still recruiting</li> <li>- Open-label Study Improving Compliance and Time of Treatment After Obtaining Complete Response Through a Tailored Schedule of Sonidegib in Locally Advanced Basal Cell Carcinomas</li> </ul> |
| <a href="#">A Study to Evaluate Neoadjuvant <b>Sonidegib</b> Followed by Surgery or Imiquimod in the Management of <b>Basal Cell Carcinoma</b></a> | Exclusion | <ul style="list-style-type: none"> <li>- Still recruiting</li> <li>- Sonidegib as neoadjuvant therapy followed by surgery or imiquimod in aBCC</li> </ul>  |
| <a href="#">Anti-PD1-antibody and Pulsed HHI for Advanced BCC</a>  | Exclusion | <ul style="list-style-type: none"> <li>- Still recruiting</li> <li>- Prospective single-arm trial with anti-PD1 antibody and pulsed hedgehog pathway inhibitor therapy in aBCC</li> </ul>  |
| <a href="#">An East Asian Study of <b>LDE225</b></a>   | Exclusion | <ul style="list-style-type: none"> <li>- Open-label dose-escalation study of sonidegib in advanced solid tumors</li> <li>*Results available</li> </ul>   |
| <a href="#">Dose Finding and Safety of Oral <b>LDE225</b> in Patients With Advanced Solid Tumors</a>   | Exclusion | <ul style="list-style-type: none"> <li>- Open-label dose-escalation study of sonidegib in advanced solid tumors</li> <li>- Completion in 2013, no results available</li> </ul>   |

BCNS = basal cell nevus syndrome BCC = basal cell carcinoma laBCC = locally advanced basal cell carcinoma aBCC = advanced basal cell carcinoma mBCC = metastatic basal cell carcinoma

**Other terms**

Saridegib

Saridegib

Saridegib

Patidegib

Patidegib

Patidegib

IPI-926

IPI-926

IPI-926

**Condition/disease**

basal cell nevus syndrome → 5 results

multiple basal cell carcinomas → 5 results, 5 duplicates

basal cell carcinoma → 6 results, 5 duplicates

basal cell nevus syndrome → 5 results, 5 duplicates

multiple basal cell carcinomas → 5 results, 5 duplicates

basal cell carcinoma → 6 results, 6 duplicates

basal cell nevus syndrome → 5 results, 5 duplicates

multiple basal cell carcinomas → 5 results, 5 duplicates

basal cell carcinoma → 6 results, 6 duplicates

**n=6 unique results, n= 1 inclusions**

| Study  | Inclusion or exclusion | Reason   |
|--|------------------------|--|
| <a href="#">Extension Study of Patidegib Topical Gel, 2% in Subjects With Gorlin Syndrome (Basal Cell Nevus Syndrome)</a>  | Exclusion              | <ul style="list-style-type: none"> <li>- Terminated due to low blinded event rate and not related to safety of the drug</li> <li>- No results available</li> </ul>   |
| <a href="#">Study of Patidegib Topical Gel, 2%, for the Reduction of Disease Burden of Persistently Developing Basal Cell Carcinomas (BCCs) in Subjects With Basal Cell Nevus Syndrome (Gorlin Syndrome)</a> | Exclusion              | <ul style="list-style-type: none"> <li>- Completion in 2020, results not available</li> <li>- Randomized vehicle-controlled trial of patidegib topical gel for multiple BCC in BCNS patients</li> </ul>                  |
| <a href="#">Trial of Patidegib Gel 2%, 4%, and Vehicle to Decrease the Number of Surgically Eligible Basal Cell Carcinomas in Gorlin Syndrome Patients</a>   | Inclusion              | Randomized vehicle-controlled trial that evaluates efficacy and safety of patidegib 2% and 4% for multiple BCC in BCNS patients  |
| <a href="#">Clinical Trial of Patidegib Gel 2%, 4%, and Vehicle Applied Once or Twice Daily to Decrease the GLI1 Biomarker in Sporadic Nodular Basal Cell Carcinomas</a>                                     | Exclusion              | <ul style="list-style-type: none"> <li>- Outcomes focuses on a maximum of one or two nodular BCCs in patients without BCNS</li> </ul>  |
| <a href="#">An Study of Patidegib Topical Gel, 2%, for the Reduction of Disease Burden of Persistently Developing Basal Cell Carcinomas in Patients With Non-Gorlin High Frequency BCC</a>                   | Exclusion              | <ul style="list-style-type: none"> <li>- Terminated due to low blinded event rate</li> <li>- No results available</li> </ul>   |
| <a href="#">IPI-926 Extension Protocol for Continuation of Treatment With IPI-926</a>  | Exclusion              | <ul style="list-style-type: none"> <li>- Oral IPI-926 extension study for patients experiencing clinical benefit from oral IPI-926 for chondrosarcoma or basal cell carcinoma</li> <li>- No results available</li> </ul> |

**Other terms**

Itraconazole

Itraconazole

Itraconazole

**n=5 unique results, n= 1 inclusions****Condition/disease**

basal cell nevus syndrome → 1 result

multiple basal cell carcinomas → 1 results, 1 duplicate

basal cell carcinoma → 6 results, 2 duplicates

| Study   | Inclusion or exclusion | Reason  |
|---|------------------------|---|
| <a href="#">Open-label Trial of SUBA™- Itraconazole (SUBA-Cap) in Subjects With Basal Cell Carcinoma Nevus Syndrome (BCCNS)</a> | Exclusion              | <ul style="list-style-type: none"> <li>- Results submitted in 2020 but not posted on clinical trials, pending after review</li> <li>- Open-label study evaluating oral SUBA-cap in patients with at least one BCC and BCNS</li> </ul> |
| <a href="#">Topical Itraconazole in the Treatment of Basal Cell Carcinoma</a>   | Exclusion              | <ul style="list-style-type: none"> <li>- Completion 2019, no results available</li> <li>- Studies molecular effects of topical itraconazole on growth of BCCs</li> </ul>  |
| <a href="#">Topical Itraconazole in Treating Patients With Basal Cell Cancer</a>  | Inclusion              | Randomized vehicle-controlled trial on topical itraconazole in patients with at least 4 BCCs  |
| <a href="#">Arsenic Trioxide and Itraconazole in Treating Patients With Advanced Basal Cell Cancer</a>                          | Exclusion              | <ul style="list-style-type: none"> <li>- Withdrawn due to logistics, no results available</li> <li>- Combination therapy for aBCC</li> </ul>  |
| <a href="#">Use Of Oral Itraconazole In Patients With Locally Limited Basocellular Carcinoma Of Skin.</a>                       | Exclusion              | <ul style="list-style-type: none"> <li>- Study status unknown</li> <li>- Open label trial on oral itraconazole prior to curative surgery for at least one localized BCC &lt;10mm</li> </ul>   |

**Other terms**

BMS-833923

BMS-833923

BMS-833923

**n=2 unique results, n= 0 inclusions****Condition/disease**

basal cell nevus syndrome → 1 result

multiple basal cell carcinomas → 2 results, 1 duplicate

basal cell carcinoma → 1 result, 1 duplicate

| Study  | Inclusion or exclusion | Reason   |
|--|------------------------|--|
| <a href="#">Study of BMS-833923 in Two Specific Patients With Basal Cell Nevus Syndrome</a>          | Exclusion              | <ul style="list-style-type: none"> <li>- Completion 2017, no results available</li> <li>- Two patients with BCNS who continue to receive BMS-833923 because of clinical benefit</li> </ul> |
| <a href="#">A Phase 1 Study of BMS-833923 (XL139) in Subjects With Advanced or Metastatic Cancer</a> | Exclusion              | <ul style="list-style-type: none"> <li>- Completion 2014, no results available</li> <li>- Outcomes focuses on patients with advanced or metastatic solid tumours</li> </ul>                |



**Other terms**

LEQ506

LEQ506

LEQ506

**n=1 unique result, n= 0 inclusions****Condition/disease**

basal cell nevus syndrome → 0 result

multiple basal cell carcinomas → 1 result

basal cell carcinoma → 1 result, 1 duplicate

| Study   | Inclusion or exclusion | Reason  |
|---|------------------------|---|
| <a href="#">A Dose Finding and Safety Study of Oral LEQ506 in Patients With Advanced Solid Tumors</a> | Exclusion              | <ul style="list-style-type: none"> <li>- Completion 2015, no results available</li> <li>- Outcomes focuses on patients with advanced or metastatic solid tumours</li> </ul> |

**Other terms**

TAK-441

TAK-441

TAK-441

**n=1 unique result, n= 0 inclusions****Condition/disease**

basal cell nevus syndrome → 0 result

multiple basal cell carcinomas → 0 result

basal cell carcinoma → 1 result

| Study  | Inclusion or exclusion | Reason  |
|--|------------------------|---|
| <a href="#">A Study of TAK-441 in Adult Patients With Advanced Nonhematologic Malignancies</a> | Exclusion              | <ul style="list-style-type: none"> <li>- Completion 2012, no results available</li> <li>- Outcomes focuses on patients with advanced or metastatic solid tumours</li> </ul> |

## Results of searches on PubMed – 17 September 2021

("HhAntag691" [Supplementary Concept]) AND "Carcinoma, Basal Cell"[Mesh] → 312 results

N=11 duplicates, N=301 unique results, n=258 excluded after screening, n=

### N= inclusions

1: Stratigos AJ, Sekulic A, Peris K, Bechter O, Prey S, Kaatz M, Lewis KD, Basset-Seguín N, Chang ALS, Dalle S, Orland AF, Licitra L, Robert C, Ulrich C, Hauschild A, Migden MR, Dummer R, Li S, Yoo SY, Mohan K, Coates E, Jankovic V, Fiaschi N, Okoye E, Bassukas ID, Loquai C, De Giorgi V, Eroglu Z, Gutzmer R, Ulrich J, Puig S, Seebach F, Thurston G, Weinreich DM, Yancopoulos GD, Lowy I, Bowler T, Fury MG. Cemiplimab in locally advanced basal cell carcinoma after hedgehog inhibitor therapy: an open-label, multi-centre, single-arm, phase 2 trial. *Lancet Oncol.* 2021 Jun;22(6):848-857. doi: 10.1016/S1470-2045(21)00126-1. Epub 2021 May 14. PMID: 34000246.

➔ Excluded after screening, research concerns cemiplimab (anti-PD1) treatment instead of hedgehog pathway inhibitor treatment

2: Kahana A, Unsworth SP, Andrews CA, Chan MP, Bresler SC, Bichakjian CK, Durham AB, Demirci H, Elner VM, Nelson CC, Kim DS, Joseph SS, Swiecicki PL, Worden FP. Vismodegib for Preservation of Visual Function in Patients with Advanced Periocular Basal Cell Carcinoma: The VISORB Trial. *Oncologist.* 2021 Jul;26(7):e1240-e1249. doi: 10.1002/onco.13820. Epub 2021 May 31. PMID: 33988881; PMCID: PMC8265335.

➔ Duplicate (clinicaltrials.gov)

3: Angnardo L, Humeda Y, Alexandraki I, Wolfe CM, Cognetta AB Jr. Vismodegib as Eye-Sparing Neoadjuvant Treatment for Locally Advanced Periocular Basal Cell Carcinoma. *J Drugs Dermatol.* 2021 May 1;20(5):552-554. doi: 10.36849/JDD.5661. PMID: 33938701.

➔ Excluded after screening, article concerns vismodegib as neoadjuvant treatment

4: Gutzmer R, Schulze HJ, Hauschild A, Leiter U, Meier F, Haferkamp S, Ulrich C, Wahl RU, Berking C, Herbst R, Häckl M, Schadendorf D. Effectiveness, safety and utilization of vismodegib in locally advanced basal cell carcinoma under real-

world conditions in Germany - The non-interventional study NIELS. *J Eur Acad Dermatol Venereol*. 2021 Aug;35(8):1678-1685. doi: 10.1111/jdv.17332. Epub 2021 May 27. PMID: 33931910.

➔ Duplicate (clinicaltrials.gov)

5: Migden M, Farberg AS, Dummer R, Squittieri N, Hanke CW. A Review of Hedgehog Inhibitors Sonidegib and Vismodegib for Treatment of Advanced Basal Cell Carcinoma. *J Drugs Dermatol*. 2021 Feb 1;20(2):156-165. doi: 10.36849/JDD.5657. PMID: 33538567.

➔ Excluded after screening, review

6: Passarelli A, Galdo G, Aieta M, Fabrizio T, Villonio A, Conca R. A Vismodegib Experience in Elderly Patients with Basal Cell Carcinoma: Case Reports and Review of the Literature. *Int J Mol Sci*. 2020 Nov 14;21(22):8596. doi: 10.3390/ijms21228596. PMID: 33202689; PMCID: PMC7696523.

➔ Excluded after eligibility assessment, 2 case reports concerned treatment of laBCC

7: Mansour KP, O'Duffy F, Webb A, Goh M, Morrison E. About face: can Vismodegib change the treatment paradigm of locally advanced basal cell carcinoma? *ANZ J Surg*. 2021 Jun;91(6):1304-1306. doi: 10.1111/ans.16399. Epub 2020 Oct 22. PMID: 33091207.

➔ Excluded after screening, article concerns treatment of laBCC

8: Villani A, Cinelli E, Fabbrocini G, Lallas A, Scalvenzi M. Hedgehog inhibitors in the treatment of advanced basal cell carcinoma: risks and benefits. *Expert Opin Drug Saf*. 2020 Dec;19(12):1585-1594. doi: 10.1080/14740338.2020.1837773. Epub 2020 Oct 22. PMID: 33054455.

➔ Excluded after screening, opinion

9: Nasifoglu S, Srour J, Lill D, Seegräber M, Sattler E, Schlaak M. Vismodegib-Therapie bei großem Basalzellkarzinom des Mittelgesichts mit Orbitabeteiligung [Vismodegib therapy for a large basal cell carcinoma on the midface with orbital involvement]. *Hautarzt*. 2020 Jul;71(Suppl 1):57-59. German. doi: 10.1007/s00105-020-04626-y. PMID: 32974719.

➔ Excluded after screening, article concerns treatment of laBCC

10: Campione E, Di Prete M, Lozzi F, Lanna C, Spallone G, Mazzeo M, Cosio T, Rapanotti C, Dika E, Gaziano R, Orlandi A, Bianchi L. High-Risk Recurrence Basal Cell Carcinoma: Focus on Hedgehog Pathway Inhibitors and Review of the Literature. *Chemotherapy*. 2020;65(1-2):2-10. doi: 10.1159/000509156. Epub 2020 Aug 10. PMID: 32777789.

➔ Excluded after screening, review

11: Dika E, Scarfi F, Ferracin M, Broseghini E, Marcelli E, Bortolani B, Campione E, Riefolo M, Ricci C, Lambertini M. Basal Cell Carcinoma: A Comprehensive Review. *Int J Mol Sci*. 2020 Aug 4;21(15):5572. doi: 10.3390/ijms21155572. PMID: 32759706; PMCID: PMC7432343.

➔ Excluded after screening, review

12: Susanto E, Marin Navarro A, Zhou L, Sundström A, van Bree N, Stantic M, Moslem M, Taylor J, Rietdijk J, Zubillaga V, Hübner JM, Weishaupt H, Wolfsberger J, Alafuzoff I, Nordgren A, Magnaldo T, Siesjö P, Johnsen JI, Kool M, Tammimies K, Darabi A, Swartling FJ, Falk A, Wilhelm M. Modeling SHH-driven medulloblastoma with patient iPS cell-derived neural stem cells. *Proc Natl Acad Sci U S A*. 2020 Aug 18;117(33):20127-20138. doi: 10.1073/pnas.1920521117. Epub 2020 Aug 3. PMID: 32747535; PMCID: PMC7443968.

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➔ Excluded after screening, dose reduction for laBCC

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➔ Excluded after screening, molecular research

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➔ Excluded after screening, quality of life during vismodegib in laBCC

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➔ Excluded after eligibility assessment does not report on reoccurrence/resistance/dosing regiment/QoL

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➔ Duplicate (clinicaltrial.gov)

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➔ Excluded after screening, clinical photo challenge

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➔ Excluded after screening, review

69: Basset-Seguin N. Traitement médical des carcinomes basocellulaires avancés: Medical treatment of advanced basal cell carcinoma. *Ann Dermatol Venereol*. 2018 Nov;145 Suppl 5:VS36-VS41. French. doi: 10.1016/S0151-9638(18)31257-2. PMID: 30477683.

➔ Excluded after screening, review

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➔ Excluded after screening, dose reduction for laBCC

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➔ Excluded after screening, neoadjuvant vismodegib for laBCC

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➔ Excluded after screening, vismodegib for periorbital laBCC

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➔ Excluded after screening, molecular research on tumor resistance

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294: Sekulic A, Migden MR, Oro AE, Dirix L, Lewis KD, Hainsworth JD, Solomon JA, Yoo S, Arron ST, Friedlander PA, Marmur E, Rudin CM, Chang AL, Low JA, Mackey HM, Yauch RL, Graham RA, Reddy JC, Hauschild A. Efficacy and safety of vismodegib in advanced basal-cell carcinoma. *N Engl J Med*. 2012 Jun 7;366(23):2171-9. doi: 10.1056/NEJMoa1113713. PMID: 22670903; PMCID: PMC5278761.

➔ Excluded after eligibility assessment, only outcomes for aBCC

295: Dlugosz A, Agrawal S, Kirkpatrick P. Vismodegib. *Nat Rev Drug Discov*. 2012 Jun 1;11(6):437-8. doi: 10.1038/nrd3753. PMID: 22653209; PMCID: PMC3383648.

➔ Excluded after screening, drug report

296: Vismodegib granted FDA approval for treatment of basal cell carcinoma. *Oncology (Williston Park)*. 2012 Feb;26(2):174, 213. PMID: 22489352.

➔ Excluded after screening, drug report

297: Allison M. Hedgehog hopes lifted by approval... and stung by failure. *Nat Biotechnol*. 2012 Mar 7;30(3):203. doi: 10.1038/nbt0312-203. PMID: 22398601.

➔ Excluded after screening, drug report

298: Tang JY. Elucidating the role of molecular signaling pathways in the tumorigenesis of basal cell carcinoma. *Semin Cutan Med Surg*. 2011 Dec;30(4 Suppl):S6-9. doi: 10.1016/j.sder.2011.11.001. PMID: 22177103.

➔ Excluded after screening, review

299: Tang JY, Marghoob AA. Emerging treatments and signaling pathway inhibitors. *Semin Cutan Med Surg*. 2011 Dec;30(4 Suppl):S14-8. doi: 10.1016/j.sder.2011.11.002. PMID: 22177102.

➔ Excluded after screening, review

300: Metcalfe C, de Sauvage FJ. Hedgehog fights back: mechanisms of acquired resistance against Smoothed antagonists. *Cancer Res*. 2011 Aug 1;71(15):5057-61. doi: 10.1158/0008-5472.CAN-11-0923. Epub 2011 Jul 19. Erratum in: *Cancer Res*. 2011 Sep 15;71(18):6087. PMID: 21771911.



➔ Excluded after screening, review

301: O'Bryan KW, Ratner D. The role of targeted molecular inhibitors in the management of advanced nonmelanoma skin cancer. *Semin Cutan Med Surg.* 2011 Mar;30(1):57-61. doi: 10.1016/j.sder.2011.01.004. PMID: 21540021.

➔ Excluded after screening, review

302: Camp WL, Turnham JW, Athar M, Elmetts CA. New agents for prevention of ultraviolet-induced nonmelanoma skin cancer. *Semin Cutan Med Surg.* 2011 Mar;30(1):6-13. doi: 10.1016/j.sder.2011.01.003. PMID: 21540016; PMCID: PMC3488433.

➔ Excluded after screening, review

303: Goldberg LH, Landau JM, Moody MN, Kazakevich N, Holzer AM, Myers A. Resolution of odontogenic keratocysts of the jaw in basal cell nevus syndrome with GDC-0449. *Arch Dermatol.* 2011 Jul;147(7):839-41. doi: 10.1001/archdermatol.2011.50. Epub 2011 Mar 21. PMID: 21422324.

➔ Excluded after screening, reports on response of odontogenic keratocysts to vismodegib

304: Watson S, Serrate C, Vignot S. Voie de signalisation Sonic Hedgehog : du développement embryonnaire aux thérapies moléculaires ciblées [Sonic Hedgehog signaling pathway: from embryology to molecular targeted therapies]. *Bull Cancer.* 2010 Dec;97(12):1477-83. French. doi: 10.1684/bdc.2010.1231. PMID: 21220225.

➔ Excluded after screening, review

305: Low JA, de Sauvage FJ. Clinical experience with Hedgehog pathway inhibitors. *J Clin Oncol.* 2010 Dec 20;28(36):5321-6. doi: 10.1200/JCO.2010.27.9943. Epub 2010 Nov 1. PMID: 21041712.

➔ Excluded after screening, review

306: Amin SH, Tibes R, Kim JE, Hybarger CP. Hedgehog antagonist GDC-0449 is effective in the treatment of advanced basal cell carcinoma. *Laryngoscope.* 2010

Dec;120(12):2456-9. doi: 10.1002/lary.21145. PMID: 20927781.

➔ Excluded after screening, vismodegib for laBCC

307: Vidal V. Les inhibiteurs de la voie Hedgehog : un espoir pour le traitement des carcinomes basocellulaires [Inhibitors of the Hedgehog signalling pathway: hope for the treatment of basal cell carcinoma]. Med Sci (Paris). 2010 Mar;26(3):231-3. French. doi: 10.1051/medsci/2010263231. PMID: 20346268.

➔ Excluded after screening, editorial

308: Dierks C. GDC-0449--targeting the hedgehog signaling pathway. Recent Results Cancer Res. 2010;184:235-8. doi: 10.1007/978-3-642-01222-8\_17. PMID: 20072843.

➔ Excluded after screening, review

309: Kean S. Medicine. Disrupting Hedgehog may reverse advanced cancer, if only temporarily. Science. 2009 Sep 4;325(5945):1188. doi: 10.1126/science.325\_1188. PMID: 19729622.

➔ Excluded after screening, reports on resistance in aBCC

310: Dlugosz AA, Talpaz M. Following the hedgehog to new cancer therapies. N Engl J Med. 2009 Sep 17;361(12):1202-5. doi: 10.1056/NEJMe0906092. Epub 2009 Sep 2. PMID: 19726764.

➔ Excluded after screening, editorial

311: Von Hoff DD, LoRusso PM, Rudin CM, Reddy JC, Yauch RL, Tibes R, Weiss GJ, Borad MJ, Hann CL, Brahmer JR, Mackey HM, Lum BL, Darbonne WC, Marsters JC Jr, de Sauvage FJ, Low JA. Inhibition of the hedgehog pathway in advanced basal-cell carcinoma. N Engl J Med. 2009 Sep 17;361(12):1164-72. doi: 10.1056/NEJMoa0905360. Epub 2009 Sep 2. PMID: 19726763.

➔ Excluded after eligibility assessment, only outcomes for aBCC

312: Robarge KD, Brunton SA, Castanedo GM, Cui Y, Dina MS, Goldsmith R, Gould SE, Guichert O, Gunzner JL, Halladay J, Jia W, Khojasteh C, Koehler MF, Kotkow

K, La H, Lalonde RL, Lau K, Lee L, Marshall D, Marsters JC Jr, Murray LJ, Qian C, Rubin LL, Salphati L, Stanley MS, Stibbard JH, Sutherlin DP, Ubhayaker S, Wang S, Wong S, Xie M. GDC-0449-a potent inhibitor of the hedgehog pathway. *Bioorg Med Chem Lett*. 2009 Oct 1;19(19):5576-81. doi: 10.1016/j.bmcl.2009.08.049. Epub 2009 Aug 15. Erratum in: *Bioorg Med Chem Lett*. 2010 Jan 15;20(2):771. PMID: 19716296.

➔ Excluded after screening, erratum

**("HhAntag691" [Supplementary Concept]) AND "Basal Cell Nevus Syndrome"[Mesh] ➔ 43 results  
N=0 unique results, n= 0 inclusions**

**("Basal cell carcinoma, multiple" [Supplementary Concept]) AND "HhAntag691" [Supplementary Concept] ➔ 6 results**

**N= 0 unique results, n= 0 inclusions**

**("sonidegib" [Supplementary Concept]) AND "Carcinoma, Basal Cell"[Mesh] ➔ 62 results**

**N=32 duplicates, N= 30 unique results, n= inclusions**

1: Fania L, Dellambra E, Moretta G, Grilli E, Di Rocco CZ, Morelli FM, Zappalà AR, Abeni D, Morese R. Efficacy of sonidegib for basal cell carcinoma in a patient affected by multiple infectious diseases. *Dermatol Ther*. 2021 Jul;34(4):e14969. doi: 10.1111/dth.14969. Epub 2021 May 9. PMID: 33928734.

➔ Excluded after eligibility assessment, only reported on laBCC outcomes

2: Moscarella E, Brancaccio G, Briatico G, Ronchi A, Verolino P, Argenziano G, Alfano R. Management of advanced basal cell carcinoma: Real-life data with sonidegib. *Dermatol Ther*. 2021 May;34(3):e14948. doi: 10.1111/dth.14948. Epub 2021 Mar 23. PMID: 33728757.

➔ Excluded after eligibility assessment, only reported on laBCC outcomes

3: Villani A, Fabbrocini G, Costa C, Scalvenzi M. Response to "Efficacy of sonidegib in histologic subtypes of advanced basal cell carcinoma: Results from the final analysis of the randomized phase 2 Basal Cell Carcinoma Outcomes with LDE225 Treatment (BOLT) trial at 42 months". *J Am Acad Dermatol*. 2021

Jun;84(6):e299-e300. doi: 10.1016/j.jaad.2021.02.074. Epub 2021 Mar 4. PMID: 33677004.

➔ Excluded after eligibility assessment, only reported on laBCC outcomes

5: Hoffmann V, Husak R, Maiwirth F, Sasama B, Zahn A, Guski S, Peitsch WK. Sonidegib in a patient with multiple basal cell carcinomas and HIV infection. *J Dtsch Dermatol Ges.* 2021 Apr;19(4):592-594. doi: 10.1111/ddg.14355. Epub 2021 Jan 14. PMID: 33448149.

➔ Included after eligibility assessment, case report on sonidegib for patient with multiple basal cell carcinomas

6: Dummer R, Lear JT, Guminski A, Leow LJ, Squittieri N, Migden M. Efficacy of sonidegib in histologic subtypes of advanced basal cell carcinoma: Results from the final analysis of the randomized phase 2 Basal Cell Carcinoma Outcomes With LDE225 Treatment (BOLT) trial at 42 months. *J Am Acad Dermatol.* 2021 Apr;84(4):1162-1164. doi: 10.1016/j.jaad.2020.08.042. Epub 2020 Dec 24. PMID: 33358380.

➔ Excluded after eligibility assessment, no outcomes reported on multiple BCC/BCNS

7: Villani A, Costa C, Fabbrocini G, Ruggiero A, Scalvenzi M. Dose reduction during routine treatment of locally advanced basal cell carcinoma with the hedgehog inhibitor sonidegib to manage adverse effects: A retrospective case series. *J Am Acad Dermatol.* 2021 Apr;84(4):e211-e212. doi: 10.1016/j.jaad.2020.12.006. Epub 2020 Dec 7. PMID: 33301802.

➔ Excluded after screening, sonidegib for laBCC

8: Sanmartín O, Llombart B, Carretero Hernández G, Flórez Menéndez Á, Botella-Estrada R, Herrera Ceballos E, Puig S. Sonidegib in the Treatment of Locally Advanced Basal Cell Carcinoma. *Actas Dermosifiliogr (Engl Ed).* 2021 Apr;112(4):295-301. English, Spanish. doi: 10.1016/j.ad.2020.11.002. Epub 2020 Nov 13. PMID: 33197438.

➔ Excluded after screening, sonidegib for laBCC

9: Conforti C, Giuffrida R, Di Meo N, Zalaudek I. Management of locally advanced basal cell carcinoma treated with sonidegib: The experience of an Italian reference hospital. *Dermatol Ther.* 2020 Nov;33(6):e14511. doi: 10.1111/dth.14511. Epub 2020 Nov 17. PMID: 33166006.

➔ Excluded after screening, sonidegib for laBCC

11: Villani A, Fabbrocini G, Costa C, Scalvenzi M. Complete remission of an advanced basal cell carcinoma after only 3-month treatment with sonidegib: Report of a case and drug management during COVID-19 pandemic. *Dermatol Ther.* 2020 Nov;33(6):e14200. doi: 10.1111/dth.14200. Epub 2020 Sep 14. PMID: 32870541.

➔ Excluded after screening, sonidegib for laBCC

14: Hou X, Rokohl AC, Ortmann M, Heindl LM. Effective treatment of locally advanced periocular basal cell carcinoma with oral hedgehog pathway inhibitor? *Graefes Arch Clin Exp Ophthalmol.* 2020 Oct;258(10):2335-2337. doi: 10.1007/s00417-020-04779-5. Epub 2020 Jun 9. PMID: 32514773; PMCID: PMC7550312.

➔ Excluded after screening, case report on sonidegib for laBCC

17: Fife K. Hedgehog pathway inhibitors come of age. *Br J Dermatol.* 2020 Jun;182(6):1322-1323. doi: 10.1111/bjd.18737. Epub 2019 Dec 17. PMID: 31849041.

➔ Excluded after screening, commentary

19: Dummer R, Guminksi A, Gutzmer R, Lear JT, Lewis KD, Chang ALS, Combemale P, Dirix L, Kaatz M, Kudchadkar R, Loquai C, Plummer R, Schulze HJ, Stratigos AJ, Trefzer U, Squittieri N, Migden MR. Long-term efficacy and safety of sonidegib in patients with advanced basal cell carcinoma: 42-month analysis of the phase II randomized, double-blind BOLT study. *Br J Dermatol.* 2020 Jun;182(6):1369-1378. doi: 10.1111/bjd.18552. Epub 2019 Dec 8. PMID: 31545507; PMCID: PMC7318253.

➔ Excluded after eligibility assessment, no outcomes reported on multiple BCC/BCNS

27: Gupta AK, Mays RR, Abramovits W, Vincent KD. Odomzo<sup>®</sup> (Sonidegib). *Skinmed.* 2018 Feb 1;16(1):35-38. PMID: 29551110.

➔ Excluded after screening, approval publication

28: Tran DC, Moffat A, Brotherton R, Pague A, Zhu GA, Chang ALS. An exploratory open-label, investigator-initiated study to evaluate the efficacy and safety of combination sonidegib and buparlisib for advanced basal cell carcinomas. *J Am Acad Dermatol*. 2018 May;78(5):1011-1013.e3. doi: 10.1016/j.jaad.2017.11.031. Epub 2017 Nov 23. PMID: 29175429.

➔ Duplicate (clinicaltrials.gov)

29: Kumari A, Ermilov AN, Grachtchouk M, Dlugosz AA, Allen BL, Bradley RM, Mistretta CM. Recovery of taste organs and sensory function after severe loss from Hedgehog/Smoothed inhibition with cancer drug sonidegib. *Proc Natl Acad Sci U S A*. 2017 Nov 28;114(48):E10369-E10378. doi: 10.1073/pnas.1712881114. Epub 2017 Nov 13. PMID: 29133390; PMCID: PMC5715770.

➔ Excluded after eligibility assessment, research on cells/molecular research

30: Chen L, Aria AB, Silapunt S, Lee HH, Migden MR. Treatment of advanced basal cell carcinoma with sonidegib: perspective from the 30-month update of the BOLT trial. *Future Oncol*. 2018 Mar;14(6):515-525. doi: 10.2217/fon-2017-0457. Epub 2017 Nov 9. PMID: 29119833.

➔ Excluded after eligibility assessment, no outcomes reported on multiple BCC/BCNS

31: Shord SS, Casey D, Zhao H, Demko S, Keegan P, Pazdur R. FDA Approval Summary: Sonidegib-Response. *Clin Cancer Res*. 2017 Oct 1;23(19):5994. doi: 10.1158/1078-0432.CCR-17-2135. PMID: 28972086.

➔ Excluded after screening, approval publication

32: Gyawali B, Ando Y. FDA Approval Summary: Sonidegib-Letter. *Clin Cancer Res*. 2017 Oct 1;23(19):5993. doi: 10.1158/1078-0432.CCR-17-1460. PMID: 28972085.

➔ Excluded after screening, approval publication

34: Lear JT, Migden MR, Lewis KD, Chang ALS, Guminski A, Gutzmer R, Dirix L, Combemale P, Stratigos A, Plummer R, Castro H, Yi T, Mone M, Zhou J, Trefzer U,

Kaatz M, Loquai C, Kudchadkar R, Sellami D, Dummer R. Long-term efficacy and safety of sonidegib in patients with locally advanced and metastatic basal cell carcinoma: 30-month analysis of the randomized phase 2 BOLT study. *J Eur Acad Dermatol Venereol*. 2018 Mar;32(3):372-381. doi: 10.1111/jdv.14542. Epub 2017 Nov 6. PMID: 28846163; PMCID: PMC5873455.

➔ Excluded after eligibility assessment, no outcomes reported on multiple BCC/BCNS

37: Casey D, Demko S, Shord S, Zhao H, Chen H, He K, Putman A, Helms W, Keegan P, Pazdur R. FDA Approval Summary: Sonidegib for Locally Advanced Basal Cell Carcinoma. *Clin Cancer Res*. 2017 May 15;23(10):2377-2381. doi: 10.1158/1078-0432.CCR-16-2051. Epub 2017 Jan 10. PMID: 28073840.

➔ Excluded after screening, approval publication

38: Shokeen D. Update on new drugs in dermatology. *Cutis*. 2016 Nov;98(5):E26-E27. PMID: 28040823.

➔ Excluded after screening, approval publication

39: Sonidegib (Odomzo®) and extensive basal cell carcinoma. *Prescrire Int*. 2017 Jan;26(178):14-15. PMID: 30730637.

➔ Excluded after screening, approval publication

40: Collier NJ, Ali FR, Lear JT. The safety and efficacy of sonidegib for the treatment of locally advanced basal cell carcinoma. *Expert Rev Anticancer Ther*. 2016 Oct;16(10):1011-8. doi: 10.1080/14737140.2016.1230020. Epub 2016 Sep 22. PMID: 27636236.

➔ Excluded after screening, review

41: Ramelyte E, Amann VC, Dummer R. Sonidegib for the treatment of advanced basal cell carcinoma. *Expert Opin Pharmacother*. 2016 Oct;17(14):1963-8. doi: 10.1080/14656566.2016.1225725. Epub 2016 Aug 29. PMID: 27538055.

➔ Excluded after screening, review

43: Tibes R. Sonidegib phosphate: new approval for basal cell carcinoma. *Drugs*

Today (Barc). 2016 May;52(5):295-303. doi: 10.1358/dot.2016.52.5.2470697. PMID: 27376162.

➔ Excluded after screening, approval publication

44: Chen L, Silapunt S, Migden MR. Sonidegib for the treatment of advanced basal cell carcinoma: a comprehensive review of sonidegib and the BOLT trial with 12-month update. *Future Oncol*. 2016 Sep;12(18):2095-105. doi: 10.2217/fon-2016-0118. Epub 2016 May 18. PMID: 27189494.

➔ Excluded after screening, review

46: Dummer R, Guminski A, Gutzmer R, Dirix L, Lewis KD, Combemale P, Herd RM, Kaatz M, Loquai C, Stratigos AJ, Schulze HJ, Plummer R, Gogov S, Pallaud C, Yi T, Mone M, Chang AL, Cornélis F, Kudchadkar R, Trefzer U, Lear JT, Sellami D, Migden MR. The 12-month analysis from Basal Cell Carcinoma Outcomes with LDE225 Treatment (BOLT): A phase II, randomized, double-blind study of sonidegib in patients with advanced basal cell carcinoma. *J Am Acad Dermatol*. 2016 Jul;75(1):113-125.e5. doi: 10.1016/j.jaad.2016.02.1226. Epub 2016 Apr 7. PMID: 27067394.

➔ Excluded after eligibility assessment, no outcomes reported on multiple BCC/BCNS

47: Sonidegib (Odomzo) for basal cell carcinoma. *Med Lett Drugs Ther*. 2016 Feb 29;58(1489):31-2. PMID: 26938701.

➔ Excluded after screening, approval publication

49: Burness CB, Scott LJ. Sonidegib: A Review in Locally Advanced Basal Cell Carcinoma. *Target Oncol*. 2016 Apr;11(2):239-46. doi: 10.1007/s11523-016-0418-9. PMID: 26867946.

➔ Excluded after screening, review

54: Burness CB. Sonidegib: First Global Approval. *Drugs*. 2015 Sep;75(13):1559-66. doi: 10.1007/s40265-015-0458-y. PMID: 26323341.

➔ Excluded after screening, approval publication



55: Migden MR, Guminski A, Gutzmer R, Dirix L, Lewis KD, Combemale P, Herd RM, Kudchadkar R, Trefzer U, Gogov S, Pallaud C, Yi T, Mone M, Kaatz M, Loquai C, Stratigos AJ, Schulze HJ, Plummer R, Chang AL, Cornélis F, Lear JT, Sellami D, Dummer R. Treatment with two different doses of sonidegib in patients with locally advanced or metastatic basal cell carcinoma (BOLT): a multicentre, randomised, double-blind phase 2 trial. *Lancet Oncol.* 2015 Jun;16(6):716-28. doi: 10.1016/S1470-2045(15)70100-2. Epub 2015 May 14. PMID: 25981810.

➔ Excluded after eligibility assessment, no reported outcomes on multiple BCC/BCNS

59: Rodon J, Tawbi HA, Thomas AL, Stoller RG, Turtschi CP, Baselga J, Sarantopoulos J, Mahalingam D, Shou Y, Moles MA, Yang L, Granvil C, Hurh E, Rose KL, Amakye DD, Dummer R, Mita AC. A phase I, multicenter, open-label, first-in-human, dose-escalation study of the oral smoothed inhibitor Sonidegib (LDE225) in patients with advanced solid tumors. *Clin Cancer Res.* 2014 Apr 1;20(7):1900-9. doi: 10.1158/1078-0432.CCR-13-1710. Epub 2014 Feb 12. PMID: 24523439.

➔ Duplicate (clinicaltrials.gov)

62: Skvara H, Kalthoff F, Meingassner JG, Wolff-Winiski B, Aschauer H, Kelleher JF, Wu X, Pan S, Mickel L, Schuster C, Sary G, Jalili A, David OJ, Emotte C, Antunes AM, Rose K, Decker J, Carlson I, Gardner H, Stuetz A, Bertolino AP, Stingl G, De Rie MA. Topical treatment of Basal cell carcinomas in nevoid Basal cell carcinoma syndrome with a smoothed inhibitor. *J Invest Dermatol.* 2011 Aug;131(8):1735-44. doi: 10.1038/jid.2011.48. Epub 2011 Mar 24. PMID: 21430703.

➔ Duplicate (clinicaltrials.gov) included

**("Basal Cell Nevus Syndrome"[Mesh]) AND "sonidegib" [Supplementary Concept] → 2 results**

**N= 2 duplicates, n=0 unique results, n=0 inclusions**

**("sonidegib" [Supplementary Concept]) AND "Basal cell carcinoma, multiple" [Supplementary Concept] → 1 result**

**N=1 duplicate, n= 0 unique results, n=0 inclusions**

**("IPI-926" [Supplementary Concept]) AND "Carcinoma, Basal Cell"[Mesh] → 1 result**

**N=1 duplicate, n= 0 unique results, n=0 inclusions**

**"IPI-926" [Supplementary Concept] AND "Basal cell carcinoma, multiple" [Supplementary Concept] → 0 results**

**("Basal Cell Nevus Syndrome"[Mesh]) AND "IPI-926" [Supplementary Concept] → 1 result**

**N=1 duplicate, n= 0 unique results, n=0 inclusions**

**("Itraconazole"[Mesh]) AND "Carcinoma, Basal Cell"[Mesh] → 13 results**

**N= 6 duplicates, n=7 unique results, n= inclusions**

1: Ciężżyńska M, Narbutt J, Skibińska M, Lesiak A. Itraconazole-A New Player in the Therapy of Advanced Basal Cell Carcinoma: A Case Report. JCO Oncol Pract. 2020 Dec;16(12):837-838. doi: 10.1200/OP.20.00273. Epub 2020 Aug 19. PMID: 32813585.

➔ Excluded after screening, itraconazole for laBCC

2: D'Arcy ME, Pfeiffer RM, Rivera DR, Hess GP, Cahoon EK, Arron ST, Brownell I, Cowen EW, Israni AK, Triplett MA, Yanik EL, Engels EA. Voriconazole and the Risk of Keratinocyte Carcinomas Among Lung Transplant Recipients in the United States. JAMA Dermatol. 2020 Jul 1;156(7):772-779. doi: 10.1001/jamadermatol.2020.1141. PMID: 32401271; PMCID: PMC7221851.

➔ Excluded after screening, different population

4: Gupta H, Tankhiwale SS. A case of bilateral eyelid histoplasmosis mistaken as basal cell carcinoma. Can J Ophthalmol. 2017 Apr;52(2):e45-e46. doi:

10.1016/j.jcjo.2016.11.030. Epub 2017 Jan 10. PMID: 28457299.

➔ Excluded after screening, different disease

7: Piérard-Franchimont C, Hermanns-Lê T, Paquet P, Herfs M, Delvenne P, Piérard GE. Hedgehog- and mTOR-targeted therapies for advanced basal cell carcinomas. *Future Oncol.* 2015 Nov;11(22):2997-3002. doi: 10.2217/fon.15.181. Epub 2015 Oct 5. PMID: 26437034.

➔ Excluded after screening, review

9: Dirix L. Discovery and exploitation of novel targets by approved drugs. *J Clin Oncol.* 2014 Mar 10;32(8):720-1. doi: 10.1200/JCO.2013.53.7118. Epub 2014 Feb 3. PMID: 24493724.

➔ Excluded after screening, editorial

10: Kim DJ, Kim J, Spaunhurst K, Montoya J, Khodosh R, Chandra K, Fu T, Gilliam A, Molgo M, Beachy PA, Tang JY. Open-label, exploratory phase II trial of oral itraconazole for the treatment of basal cell carcinoma. *J Clin Oncol.* 2014 Mar 10;32(8):745-51. doi: 10.1200/JCO.2013.49.9525. Epub 2014 Feb 3. PMID: 24493717.

➔ Duplicate (clinicaltrials.gov), included

12: Kim J, Tang JY, Gong R, Kim J, Lee JJ, Clemons KV, Chong CR, Chang KS, Fereshteh M, Gardner D, Reya T, Liu JO, Epstein EH, Stevens DA, Beachy PA. Itraconazole, a commonly used antifungal that inhibits Hedgehog pathway activity and cancer growth. *Cancer Cell.* 2010 Apr 13;17(4):388-99. doi: 10.1016/j.ccr.2010.02.027. PMID: 20385363; PMCID: PMC4039177.

➔ Excluded after eligibility assessment, cell research

13: Manohar A, Nizlan MN. Chronic nonhealing ulcer of the right thumb with multiple subcutaneous nodules. *Orthopedics.* 2008 Jul;31(7):710. PMID: 19292371.

➔ Excluded after screening, different disease

**("Basal Cell Nevus Syndrome"[Mesh]) AND "Itraconazole"[Mesh] ➔ 0 results  
N= 0 unique results, n=0 inclusions**

("Itraconazole"[Mesh]) AND "Basal cell carcinoma, multiple" [Supplementary Concept] → 0 results  
N= 0 unique results, n=0 inclusions

("Carcinoma, Basal Cell"[Mesh]) AND "BMS-833923" [Supplementary Concept] → 2 results  
N=2 duplicates, n= 0 unique results, n=0 inclusions

("BMS-833923" [Supplementary Concept]) AND "Basal Cell Nevus Syndrome"[Mesh] → 1 result  
N= 0 unique results, n=0 inclusions

("Basal cell carcinoma, multiple" [Supplementary Concept]) AND "BMS-833923" [Supplementary Concept] → 0 results  
N= 0 unique results, n=0 inclusions

("Carcinoma, Basal Cell"[Mesh]) AND "NVP-LEQ506" [Supplementary Concept] → 0 results  
N= 0 unique results, n=0 inclusions

("NVP-LEQ506" [Supplementary Concept]) AND "Basal Cell Nevus Syndrome"[Mesh] → 0 results  
N= 0 unique results, n=0 inclusions

("Basal cell carcinoma, multiple" [Supplementary Concept]) AND "NVP-LEQ506" [Supplementary Concept] → 0 results  
N= 0 unique results, n=0 inclusions

("Carcinoma, Basal Cell"[Mesh]) AND "TAK-441" [Supplementary Concept] → 0 results  
N= 0 unique results, n=0 inclusions

("TAK-441" [Supplementary Concept]) AND "Basal Cell Nevus Syndrome"[Mesh] → 0 results  
N= 0 unique results, n=0 inclusions

("TAK-441" [Supplementary Concept]) AND "Basal cell carcinoma, multiple" [Supplementary Concept] → 0 results  
N= 0 unique results, n=0 inclusions

## EMBASE VISMODEGIB AND BASAL CELL CARCINOMA

Database: Embase <1974 to 2021 Week 37>

Search Strategy:

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1 basal cell carcinoma/ (28620)

2 vismodegib/ (2276)

3 1 and 2 (1074)

AND publication type: article (206)

\*\*\*\*\*

Duplicates: 66

Screened:

Assesses:

Inclusion:

1.

Hedgehog inhibitors with and without adjunctive therapy in treatment of locally advanced basal cell carcinoma.

Patel A.D., Ravichandran S., Kheterpal M.

International Journal of Dermatology. (no pagination), 2021. Date of Publication: 2021.

[Article]

Publisher

John Wiley and Sons Inc

➔ Excluded after screening, treatment of laBCC

2.

Vismodegib for treatment of periocular basal cell carcinoma - 6-year experience from a tertiary cancer center.

Xavier C., Lopes E., Bexiga C., Moura C., Gouveia E., Duarte A.F.

Anais Brasileiros de Dermatologia. (no pagination), 2021. Date of Publication: 2021.

[Article]

Publisher

Elsevier Espana S.L.

→ Excluded after screening, vismodegib for laBCC

3.

ABT-199 inhibits Hedgehog pathway by acting as a competitive inhibitor of oxysterol, rather as a BH3 mimetic.

Wang J., Zhang Y., Huang W.-J., Yang J., Tang W.-G., Huang T.-M., Tan W.-F.

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Experience with treating basal cell carcinoma of the occipital region with regional lymph node metastasis. ОблТ рМеHeH yeBo Tera B koMbHpoBaHHoM eeH ba3abHokeToHoo paka ko 3aTbloHo obacT c MeTacTa3oM B peoHapHbl MaTeck y3e <ОблТ рМеHeH yeBo Tera B koMbHpoBaHHoM eeH ba3abHokeToHoo paka ko 3aTbloHo obacT c MeTacTa3oM B peoHapHbl MaTeck y3e.>

Abramova O.E., Kudryavtsev D.V., Gumenetskaya Yu.V.

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Publisher

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Furdova A., Kapitanova K., Kollarova A., Sekac J.

Oncology Reviews. 14(1) (pp 36-42), 2020. Date of Publication: 30 Apr 2020.

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Publisher

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→ Excluded after screening, review

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Sensitivity of treatment patterns to grace period selection in medications with tolerability issues: A real-world example of hedgehog inhibitor use in basal cell carcinoma.

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Publisher

John Wiley and Sons Ltd

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Real-life data on basal cell carcinoma treatment: Insights on clinicians' therapeutic choices from an institutional hospital registry.

Manoli S.-M., Moutsoudis A., Papageorgiou C., Lallas K., Rigas H.-M., Kyrmanidou E., Papadimitriou I., Paschou E., Spyridis I., Gkentsidi T., Sotiriou E., Vakirlis E., Ioannidis D., Apalla Z., Lallas A.

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Publisher

Blackwell Publishing Inc.

➔ Excluded after eligibility assessment, no information on indication/response/qol/reoccurrence/side effects of vismodegib treatment

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Long non-coding RNAs in cutaneous biology and keratinocyte carcinomas.

Piipponen M., Nissinen L., Kahari V.-M.

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Springer Science and Business Media Deutschland GmbH

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Non-melanoma skin cancers: Biological and clinical features.

Cives M., Mannavola F., Lospalluti L., Sergi M.C., Cazzato G., Filoni E., Cavallo F., Giudice G., Stucci L.S., Porta C., Tucci M.

International Journal of Molecular Sciences. 21(15) (pp 1-24), 2020. Article Number: 15394. Date of Publication: 01 Aug 2020.

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Publisher

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➔ Excluded after screening, review on nonmelanoma skin cancers

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Lentivirus-mediated CD44s expression increases human basal cell carcinoma cell resistance against vismodegib.

Ren J., Ma X., Tu C., Li Z.

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Publisher

Spandidos Publications (10 Vriaxidos Street, Athens 116 10, Greece. E-mail: subscriptions@spandidos-publications.com)

➔ Excluded after eligibility assessment, in vitro and in vivo models on resistance to vismodegib

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Modrakowska P., Balik K., Maj M., Bajek A.



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➔ Excluded after screening, review

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Peytchinski, Gospodin Iliev ET

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[Article]

Publisher

John Libbey

➔ Excluded after screening, journal club

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The use of hedgehog antagonists in cancer therapy: a comparison of clinical outcomes and gene expression analyses.

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Publisher

Bellwether Publishing, Ltd.

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Publisher

MDPI AG

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Publisher

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➔ Excluded after screening, review

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Locally Advanced Recurrence of Periocular Squamous Cell Carcinoma and Basal Cell Carcinoma After Mohs Micrographic Surgery.

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Publisher

Elsevier Inc.

➔ Excluded after screening, review

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Metastatic basal cell carcinoma with atypical pattern of spread.

Gellatly M., Cruzval-O'Reilly E., Mervak J.E., Mervak B.M.

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Slominski A.T., Brozyna A.A., Zmijewski M.A., Janjetovic Z., Kim T.-K., Slominski R.M., Tuckey R.C., Mason R.S., Jetten A.M., Guroji P., Reichrath J., Elmets C., Athar M.

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Publisher

Springer

→ Excluded after screening, book chapter

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Publisher

BMJ Publishing Group

→ Excluded after screening, different disease

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Publisher

Nature Research

→ Excluded after screening, marker research for vismodegib resistance  
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Ahluwalia J., Avram M.M., Ortiz A.E.

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[Review]

Publisher

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→ Excluded after screening, different treatment

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Publisher

MDPI AG (E-mail: diversity@mdpi.com)

➔ Excluded after screening, review on cancer fibroblasts

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Ablative fractional laser-assisted treatments for keratinocyte carcinomas and its precursors-Clinical review and future perspectives.

Erlendsson A.M., Olesen U.H., Haedersdal M., Rossi A.M.

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Publisher

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Banvolgyi A., Lorincz K., Kiss N., Avci P., Fesus L., Szipocs R., Krenacs T., Gyongyosi N., Wikonkal N., Karpati S., Nemeth K.

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Publisher

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➔ Excluded after screening, different treatment

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Publisher

Mattioli 1885 (E-mail: [valeriaceci@mattioli1885.com](mailto:valeriaceci@mattioli1885.com))

➔ Excluded after screening, different disease

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Laageide L., Wendl E., Wadle J., Powers J.

JAAD Case Reports. 6(10) (pp 1019-1023), 2020. Date of Publication: October 2020.

[Article]

Publisher

Elsevier Inc.

➔ Excluded after screening, advanced BCC

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Enhanced and Sustained Cutaneous Delivery of Vismodegib by Ablative Fractional Laser and Microemulsion Formulation.

Olesen U.H., Clergeaud G., Hendel K.K., Yeung K., Lerche C.M., Andresen T.L., Haedersdal M.

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Publisher

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➔ Excluded after screening, combination therapy of vismodegib and laser

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➔ Excluded after screening, vismodegib for aBCC

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Ashraf D.C., Vagefi M.R.

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Publisher

Lippincott Williams and Wilkins (E-mail: [kathiest.clai@apta.org](mailto:kathiest.clai@apta.org))

➔ Excluded after screening, aBCC



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Publisher

Lippincott Williams and Wilkins (E-mail: [kathiest.clai@apta.org](mailto:kathiest.clai@apta.org))

➔ Excluded after screening, editorial

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Niebel D., Sirokay J., Hoffmann F., Frohlich A., Bieber T., Landsberg J.

Dermatology and Therapy. 10(4) (pp 835-846), 2020. Date of Publication: 01 Aug 2020.

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➔ Excluded after screening, short review

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Publisher

Springer (E-mail: [editorial@springerplus.com](mailto:editorial@springerplus.com))

→ Excluded after screening, cell research on aBCC

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Su M.G., Potts L.B., Tsai J.H.

American Journal of Ophthalmology Case Reports. 19 (no pagination), 2020. Article Number: 100755. Date of Publication: September 2020.

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Publisher

Elsevier Inc

→ Excluded after screening, vismodegib as neoadjuvant

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Publisher

Elsevier Inc

→ Excluded after screening, case report on rare adverse event

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Cohen P.R., Kurzrock R.

Journal of the American Academy of Dermatology. 82(6) (pp e253-e254), 2020. Date of Publication: June 2020.

[Letter]

Publisher

Mosby Inc. (E-mail: customerservice@mosby.com)

→ Excluded after screening, different treatment

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Low-Dose Vismodegib as Maintenance Therapy After Locally Advanced Basal Cell Carcinoma Complete Remission: High Efficacy with Minimal Toxicity.

Scalvenzi M., Cappello M., Costa C., Fabbrocini G., Luciano M., Villani A.

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Hamid A.B., Petreaca R.C.

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Publisher

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→ Excluded after screening, review

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Soebagjo H.D.

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Publisher

EManuscript Technologies (E-mail: [journals@emanuscript.in](mailto:journals@emanuscript.in))

➔ Excluded after screening, mini review

144.

Synergistic effect of anti-PD1 immunotherapy then radiotherapy in advanced basal cell carcinoma. Synergie de la sequence immunotherapie par anti-PD1 et radiotherapie au cours d'un carcinome basocellulaire avance <Synergie de la sequence immunotherapie par anti-PD1 et radiotherapie au cours d'un carcinome basocellulaire avance.>

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Annales de Dermatologie et de Venereologie. 147(3) (pp 228-232), 2020. Date of Publication: March 2020.

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Elsevier Masson SAS (62 rue Camille Desmoulins, Issy les Moulineaux Cedex 92442, France)

➔ Excluded after screening, different combination therapy

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Rudkovskaia A.A., Kazmi S.A., Gitter L., Ayers M.L.

Journal of Bronchology and Interventional Pulmonology. 27(1) (pp E8-E10), 2020. Date of Publication: 01 Jan 2020.

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Publisher

Lippincott Williams and Wilkins (E-mail: [kathiest.clai@apta.org](mailto:kathiest.clai@apta.org))

➔ Excluded after screening, mBCC

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Oudit D., Pham H., Grecu T., Hodgson C., Grant M.E., Rashed A.A., Allan D., Green A.C.

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Curragh D.S., Huilgol S.C., Selva D.

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Publisher

Springer Nature

→ Excluded after screening, neoadjuvant vismodegib for aBCC

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Shoji M.K., Pirakitikulr N., Tran A.Q., Wester S.T.

Orbit (London). (no pagination), 2020. Date of Publication: 2020.

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Publisher

Taylor and Francis Ltd

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Lee A.Y., Berman R.S.

Annals of Surgical Oncology. 27(1) (pp 22-27), 2020. Date of Publication: 01 Jan 2020.

[Review]

Publisher

Springer

➔ Excluded after screening, review

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Vismodegib, a hedgehog pathway inhibitor, causing severe liver injury.

Bui R., Strader M., Viterbo D.

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Publisher

Wolters Kluwer Health

➔ Excluded after screening, report on known, rare side effect

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18445 Basal cell carcinoma gene mutations and polymorphisms differ between Asian, Hispanic, and Caucasian patients.

Hass B., Higgins S., Lobl M., Clarey D., Sutton A.V., Wysong A.

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[Conference Abstract]

Publisher

Mosby Inc.

→ Excluded after screening, molecular research in BCC  
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Ross J.S., Sokol E.S., Pavlick D.C., Vergilio J.-A., Killian J.K., Williams E., Danziger N.A., Tse J., Ramkissoon S., Severson E., Hemmerich A., Duncan D., Huang R., Madison R., Schrock A.B., Alexander B., Venstrom J., Reddy P., McGregor K., Elvin J.

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Publisher

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→ Excluded after screening, genomic alterations

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Apalla Z., Spyridis I., Kyrgidis A., Lazaridou E., Kyriakou A., Fotiadou C., Pikou O., Sotiriou E., Vakirlis E., Papageorgiou C., Delli F., Moutsoudis A., Manoli S.M., Ioannides D., Lallas A.

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→ Excluded after eligibility assessment, does not discuss outcomes for multiple BCC patients separately

158.

The effectiveness of vismodegib in patients with advanced periocular basal cell carcinoma: a case series of 13 patients.

Villani A., Costa C., Cappello M., Fabbrocini G., Scalvenzi M.

Journal of Dermatological Treatment. (no pagination), 2020. Date of Publication: 2020.

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Publisher

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→ Excluded after screening, vismodegib for aBCC

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Schnetzler G., Cuberos M., Bucher R., Stassen K., Mingrino R.

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[Article]

Publisher

Taylor and Francis Ltd

→ Excluded after screening, discusses pregnancy prevention

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Molecular testing in metastatic basal cell carcinoma.



Verkouteren B.J.A., Wakkee M., van Geel M., van Doorn R., Winnepenninckx V.J., Korpershoek E., Mooyaart A.L., Reyners A.K.L., Terra J.B., Aarts M.J.B., Reinders M.G.H.C., Mosterd K.

Journal of the American Academy of Dermatology. (no pagination), 2020. Date of Publication: 2020.

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Publisher

Mosby Inc. (E-mail: customerservice@mosby.com)

➔ Excluded after screening, vismodegib for mBCC

161.

Analysis of Longitudinal-Ordered Categorical Data for Muscle Spasm Adverse Event of Vismodegib: Comparison Between Different Pharmacometric Models.

Lu T., Yang Y., Jin J.Y., Kagedal M.

CPT: Pharmacometrics and Systems Pharmacology. 9(2) (pp 96-105), 2020. Date of Publication: 01 Feb 2020.

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Publisher

American Society for Clinical Pharmacology and Therapeutics

➔ Excluded after screening, pharmacometric model for known adverse event

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Targeting signalling pathways and the immune microenvironment of cancer stem cells - a clinical update.

Clara J.A., Monge C., Yang Y., Takebe N.

Nature Reviews Clinical Oncology. 17(4) (pp 204-232), 2020. Date of Publication: 01 Apr 2020.

[Review]

Publisher

Nature Research

➔ Excluded after screening, review

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S2k Guidelines for Cutaneous Basal Cell Carcinoma - Part 2: Treatment, Prevention and Follow-up.

Lang B.M., Balermipas P., Bauer A., Blum A., Brolsch G.F., Dirschka T., Follmann M., Frank J., Frerich B., Fritz K., Hauschild A., Heindl L.M., Howaldt H.-P., Ihrler S., Kakkassery V., Klumpp B., Krause-Bergmann A., Loser C., Meissner M., Sachse M.M., Schlaak M., Schon M.P., Tischendorf L., Tronnier M., Vordermark D., Welzel J., Weichenthal M., Wiegand S., Kaufmann R., Grabbe S.

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Publisher

Wiley-VCH Verlag

➔ Excluded after screening, BCC guidelines

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Tanese K.

Current Treatment Options in Oncology. 20(2) (no pagination), 2019. Article Number: 13. Date of Publication: February 2019.

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Publisher

Springer Science and Business Media, LLC

➔ Excluded after screening, BCC guidelines

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Polyamine metabolism as a therapeutic target in hedgehog-driven basal cell carcinoma and medulloblastoma.

Coni S., Magno L.D., Serrao S.M., Kanamori Y., Agostinelli E., Canettieri G.

Cells. 8(2) (no pagination), 2019. Article Number: 150. Date of Publication: February 2019.

[Review]

Publisher

MDPI AG (Postfach, Basel CH-4005, Switzerland. E-mail: indexing@mdpi.com)

→ Excluded after screening, review

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Treatment of inoperable basosquamous carcinoma with hedgehog inhibitors, vismodegib and sonedigib: A case series.

Scott J., DiLorenzo A., Chang S., Harvell J., Jang S., DeSimone J.

Journal of the American Academy of Dermatology. Conference: American Academy of Dermatology 2019 Annual Meeting. District of Columbia United States. 81(4 Supplement 1) (pp AB140), 2019. Date of Publication: October 2019.

[Conference Abstract]

Publisher

Mosby Inc.

→ Excluded after screening, hedgehog pathway inhibitors for aBCC

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Clonal replacement of tumor-specific T cells following PD-1 blockade.

Yost K.E., Satpathy A.T., Wells D.K., Qi Y., Wang C., Kageyama R., McNamara K.L., Granja J.M., Sarin K.Y., Brown R.A., Gupta R.K., Curtis C., Bucktrout S.L., Davis M.M., Chang A.L.S., Chang H.Y.

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Publisher

→ Excluded after screening, different treatment

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Harris L.

U.S. Pharmacist. 44(8) (pp 29-35), 2019. Date of Publication: August 2019.

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Publisher

Jobson Publishing Corporation

→ Excluded after screening, guideline

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Coexistence of Pemphigus Foliaceus and Grover Disease after a Radical Surgery for Basal Cell Carcinoma.

Magdaleno-Tapiál J., Valenzuela-Onate C., Martínez-Domenech A., García-Legaz-Martínez M., Carballeira-Brana A., Sánchez-Carazo J.L., Pérez-Ferriols A., Alegre-De Miquel V.

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Publisher

Lippincott Williams and Wilkins (E-mail: [kathiest.clai@apta.org](mailto:kathiest.clai@apta.org))

→ Excluded after screening, different disease

180.

Vismodegib for the treatment of radiation-induced basal cell carcinoma - a case report and brief literature study.

Laliscia C., Baldaccini D., Antonuzzo A., Paiar F.

Wspolczesna Onkologia. 23(4) (pp 251-253), 2019. Date of Publication: 2019.

- Excluded after eligibility assessment, case report on patient multiple BCCs and vismodegib, does not report on reoccurrence/resistance/dosing regiment/QoL

181.

Not everything is what it seems within geriatric dermatology-oncology. Niet alles is wat het lijkt binnen de geriatrische dermatologie <Niet alles is wat het lijkt binnen de geriatrische dermatologie.>

Kerkhof M.A.M., van Winden M.E.C., van Aalst W., Amir A., Hoeben B.A.W., Lubeek S.F.K.

Nederlands Tijdschrift voor Dermatologie en Venereologie. 29(10) (pp 14-17), 2019. Date of Publication: November 2019.

[Article]

Publisher

Stichting Beheer Tijdschriften Dermatologie (E-mail: l.fritschy@nvdv.nl)

- Excluded after screening, vismodegib for laBCC

182.

Hedgehog/GLI signaling in tumor immunity - New therapeutic opportunities and clinical implications.

Grund-Groschke S., Stockmaier G., Aberger F.

Cell Communication and Signaling. 17(1) (no pagination), 2019. Article Number: 172. Date of Publication: 26 Dec 2019.

[Review]

Publisher

BioMed Central Ltd. (E-mail: info@biomedcentral.com)

- Excluded after screening, review

186.

Complete response of a locally advanced basosquamous carcinoma with vismodegib treatment.

Sahuquillo-Torralba A., Llavador-Ros M., Caballero-Daroqui J., Botella-Estrada R.

Indian Journal of Dermatology, Venereology and Leprology. 85(5) (pp 549-552), 2019. Date of Publication: September-October 2019.

[Letter]

Publisher

Wolters Kluwer Medknow Publications (B9, Kanara Business Centre, off Link Road, Ghatkopar (E), Mumbai 400 075, India)

➔ Excluded after screening, laBCC

188.

Aging and the treatment of basal cell carcinoma.

Sreekantaswamy S., Endo J., Chen A., Butler D., Morrison L., Linos E.

Clinics in Dermatology. 37(4) (pp 373-378), 2019. Date of Publication: July - August 2019.

[Article]

Publisher

Elsevier Inc. (E-mail: usjcs@elsevier.com)

➔ Excluded after screening, opinion/review

190.

Diagnosis and treatment of basal cell carcinoma: European consensus-based interdisciplinary guidelines.

Peris K., Fagnoli M.C., Garbe C., Kaufmann R., Bastholt L., Seguin N.B., Bataille V., Marmol V.D., Dummer R., Harwood C.A., Hauschild A., Holler C., Haedersdal M., Malvehy J., Middleton M.R., Morton C.A., Nagore E., Stratigos A.J., Szeimies R.-M., Tagliaferri L., Trakatelli M., Zalaudek I., Eggermont A., Grob J.J.

European Journal of Cancer. 118 (pp 10-34), 2019. Date of Publication: September 2019.

[Article]

Publisher

Elsevier Ltd

➔ Excluded after screening, BCC guidelines

191.

Potential molecular mechanisms of the anti-cancer activity of Vitamin D.

Skrajnowska D., Bobrowska-Korczak B.

Anticancer Research. 39(7) (pp 3353-3363), 2019. Date of Publication: 2019.

[Review]

Publisher

International Institute of Anticancer Research (1st km Kapandritiou - Kalamou Rd., P.O. Box 22, Kapandriti, Attica GR-19014, Greece. E-mail: [subscriptions@iiar-anticancer.org](mailto:subscriptions@iiar-anticancer.org))

➔ Excluded after screening, review

192.

Hedgehog signaling inhibitors in solid and hematological cancers.

Cortes J.E., Gutzmer R., Kieran M.W., Solomon J.A.

Cancer Treatment Reviews. 76 (pp 41-50), 2019. Date of Publication: June 2019.

[Review]

Publisher

W.B. Saunders Ltd

➔ Excluded after screening, review

193.

Medications Associated with Increased Risk of Keratinocyte Carcinoma.

Crow L.D., Kaizer-Salk K.A., Juszcak H.M., Arron S.T.

Dermatologic Clinics. 37(3) (pp 297-305), 2019. Date of Publication: July 2019.

[Review]

Publisher

W.B. Saunders

➔ Excluded after screening, review different topic

195.

Eruptive epidermoid cysts after imiquimod treatment of recurrent basal cell carcinoma: A case report. Eruptive Epidermoidzysten nach Imiquimod-Therapie eines rezidivierenden Basalzellkarzinoms: Ein Fallbericht <Eruptive Epidermoidzysten nach Imiquimod-Therapie eines rezidivierenden Basalzellkarzinoms: Ein Fallbericht.>

Woltsche N., El-Shabrawi-Caelen L., Deinlein T., Kupsa R., Gschwandtner M., Hofmann-Wellenhof R., Zalaudek I.

Hautarzt. 70(5) (pp 363-366), 2019. Date of Publication: 01 May 2019.

[Article]

Publisher

Springer Verlag (E-mail: [service@springer.de](mailto:service@springer.de))

➔ Excluded after screening, often described adverse event after vismodegib for laBCC

200.

Combination of novel systemic agents and radiotherapy for solid tumors - part I: An AIRO (Italian association of radiotherapy and clinical oncology) overview focused on treatment efficacy.

Arcangeli S., Jereczek-Fossa B.A., Alongi F., Aristei C., Becherini C., Belgioia L., Buglione M., Caravatta L., D'Angelillo R.M., Filippi A.R., Fiore M., Genovesi D., Greco C., Livi L., Magrini S.M., Marvaso G., Mazzola R., Meattini I., Merlotti A., Palumbo I., Pergolizzi S., Ramella S., Ricardi U., Russi E., Trovo M., Sindoni A., Valentini V., Corvo R.

Critical Reviews in Oncology/Hematology. 134 (pp 87-103), 2019. Date of Publication: February 2019.

[Review]

Publisher

Elsevier Ireland Ltd



➔ Excluded after screening, review

201.

What's New in Genetic Skin Diseases.

Hill C.R., Theos A.

Dermatologic Clinics. 37(2) (pp 229-239), 2019. Date of Publication: April 2019.

[Review]

Publisher

W.B. Saunders

➔ Excluded after screening, review

202.

Therapeutic implications of cancer epithelial-mesenchymal transition (EMT).

Cho E.S., Kang H.E., Kim N.H., Yook J.I.

Archives of Pharmacal Research. 42(1) (pp 14-24), 2019. Date of Publication: 17 Jan 2019.

[Review]

Publisher

Pharmaceutical Society of Korea (E-mail: pskor@chollian.net)

➔ Excluded after screening, review

203.

Skin adverse events in recently approved targeted therapies in solid malignancies.

Habre M., Salloum A., Habre S.B., Abi Chebl J., Dib R., Kourie H.R.

Future Oncology. 15(3) (pp 331-343), 2019. Date of Publication: January 2019.

[Review]

Publisher

Future Medicine Ltd. (E-mail: info@futuremedicine.com)

→ Excluded after screening, review

204.

Extensive bony metastases from facial metatypical basal cell carcinoma: a case report.

Pabst A., Klinghuber M., Muller G., Vandersee S., Werkmeister R.

British Journal of Oral and Maxillofacial Surgery. 57(1) (pp 82-84), 2019. Date of Publication: January 2019.

[Article]

Publisher

Churchill Livingstone

→ Excluded after screening, mBCC

205.

Overexpression of Desmoglein 2 in a Mouse Model of Gorlin Syndrome Enhances Spontaneous Basal Cell Carcinoma Formation through STAT3-Mediated Gli1 Expression.

Brennan-Crispi D.M., Overmiller A.M., Tamayo-Orrego L., Marous M.R., Sahu J., McGuinn K.P., Cooper F., Georgiou I.C., Frankfurter M., Salas-Alanis J.C., Charron F., Millar S.E., Mahoney M.G., Riobo-Del Galdo N.A.

Journal of Investigative Dermatology. 139(2) (pp 300-307), 2019. Date of Publication: February 2019.

[Article]

Publisher

Elsevier B.V.

→ Excluded after screening, in vitro and in vivo studies on molecular expression

## EMBASE VISMODEGIB AND BASAL CELL NEVUS SYNDROME

Database: Embase <1974 to 2021 Week 37>

Search Strategy:

- 
- 1 vismodegib/ (2276)
  - 2 basal cell carcinoma/ (28620)
  - 4 basal cell nevus syndrome/ (2721)
  - 5 1 and 4 (236)

\*\*\*\*\*

Duplicates: 111

1.

Efficacy and tolerability of vismodegib treatment in locally advanced and metastatic basal cell carcinoma: Retrospective real-life data.

Gurbuz M., Dogan I., Akkus E., Ermis H., Utkan G., Vatansever S., Tas F.

Dermatologic Therapy. (no pagination), 2021. Date of Publication: 2021.

➔ Excluded after eligibility assessment, only reports on laCC and mBCC

2.

Sonidegib after vismodegib discontinuation in a patient with Gorlin-Goltz syndrome and multiple basal cell carcinomas.

Piccerillo A., Di Stefani A., Costantini A., Peris K.

Dermatologic Therapy. (no pagination), 2021. Date of Publication: 2021.

➔ Included after eligibility assessment, reoccurrence of bcc's after vismodegib and consequently treated with sonidegib

4.

A case study on the use of Sonidegib in Basal-nevus syndrome.

Tam L., Ladwa R.

Australasian Journal of Dermatology. Conference: 53rd Annual Scientific Meeting of the Australasian College of Dermatologists, The Art of Dermatology. Virtual. 62(SUPPL 1) (pp 78-79), 2021. Date of Publication: April 2021.

→ Excluded after eligibility assessment, case report sonidegib for BCNS does not report on reoccurrence/resistance/dosing regiment/QoL

5.

Retrospective investigation of hereditary syndromes in patients with medulloblastoma in a single institution.

Wang Y., Wu J., Li W., Li J., Liu R., Yang B., Li C., Jiang T.

Child's Nervous System. 37(2) (pp 411-417), 2021. Date of Publication: February 2021.

[Article]

Publisher

Springer Science and Business Media Deutschland GmbH

→ Excluded after screening, different disease

9.

Vismodegib hedgehog-signaling inhibition and treatment of basal cell carcinomas in gorlin-goltz syndrome.

Mendes S.R., Brinca A., Vieira R.

Journal of the Dermatology Nurses' Association. Conference: 24th World Congress of Dermatology. Milan Italy. 12(2) (no pagination), 2020. Date of Publication: March-April 2020.

→ Included after eligibility assessment, case report with mention of reoccurrence and different schedule

10.

Nevoid basal cell carcinoma syndrome: Literature review and case report.

Alifakioti D., Zafiriou E., Gravani A., Gidarokosta P., Roussaki-Schulze A.

Journal of the Dermatology Nurses' Association. Conference: 24th World Congress of Dermatology. Milan Italy. 12(2) (no pagination), 2020. Date of Publication: March-April 2020.

[Conference Abstract]

Publisher

Lippincott Williams and Wilkins

➔ Excluded after screening, review

11.

Targeted treatment for shh+ medulloblastoma in a pediatric patient with gorlin syndrome.

Johnnidis M.

Pediatric Blood and Cancer. Conference: 2020 American Society of Pediatric Hematology/Oncology Conference, ASPHO 2020. Fort Worth, TX United States. 67(SUPPL 2) (no pagination), 2020. Date of Publication: June 2020.

[Conference Abstract]

Publisher

John Wiley and Sons Inc.

➔ Excluded after screening, different disease

12.

Genodermatoses with malignant potential.

Ladd R., Davis M., Dyer J.A.

Clinics in Dermatology. 38(4) (pp 432-454), 2020. Date of Publication: July - August 2020.

[Article]

Publisher

Elsevier Inc. (E-mail: [sinfo-f@elsevier.com](mailto:sinfo-f@elsevier.com))

➔ Excluded after screening, genodermatoses in general  
19.

Photocarcinogenesis.

Subhadarshani S., Athar M., Elmets C.A.

Current Dermatology Reports. 9(3) (pp 189-199), 2020. Date of Publication: 01 Sep 2020.

[Review]

Publisher

Springer

➔ Excluded after screening, review  
22.

Efficacy and tolerability of vismodegib treatment in locally advanced and metastatic basal cell carcinoma.

Gurbuz M., Dogan I., Utkan G., Tas F.

Annals of Oncology. Conference: ESMO Asia Virtual Congress. Virtual, Online. 31(Supplement 6) (pp S1410-S1411), 2020. Date of Publication: November 2020.

[Conference Abstract]

Publisher

Elsevier Ltd

➔ Excluded after screening, vismodegib for aBCC

23.

388 Natural history and management of basal cell nevus syndrome: Updates from the gorlin syndrome registry.

Eng V., Saldanha G., Li S., Bailey-Healy I., Teng J., Tang J.

Journal of Investigative Dermatology. Conference: Society for Investigative Dermatology 2020. Virtual, Online. 140(7 Supplement) (pp S50), 2020. Date of Publication: July 2020.

→ Excluded after eligibility assessment, does not discuss hpi outcomes

26.

Treatment and therapeutic rest with vismodegib in a patient with Gorlin Goltz syndrome. Tratamiento y descanso terapeutico con vismodegib en un paciente con sindrome de Gorlin Goltz <Tratamiento y descanso terapeutico con vismodegib en un paciente con sindrome de Gorlin Goltz.>

Gonzalez Gonzalez M.A., Matilla Fernandez M.B., Ferreras Lopez N., Nieto Mangudo B., Ortiz de Urbina Gonzalez J.J.

Piel. 35(1) (pp 22-24), 2020. Date of Publication: January 2020.

→ Excluded after eligibility assessment, daily dosing for BCNS patient

William's syndrome associated with extensive infiltrative basal cell carcinoma.

Mosca M., Azzam M., Savell A., Hovenic W.

Journal of the American Academy of Dermatology. Conference: American Academy of Dermatology 2019 Annual Meeting. District of Columbia United States. 81(4 Supplement 1) (pp AB298), 2019. Date of Publication: October 2019.

[Conference Abstract]

Publisher

Mosby Inc.

→ Excluded after screening, different disease



29.

Hereditary Tumor Syndromes with Skin Involvement.

Hamid R.N., Akkurt Z.M.

Dermatologic Clinics. 37(4) (pp 607-613), 2019. Date of Publication: October 2019.

[Review]

Publisher

W.B. Saunders

→ Excluded after screening, review

36.

Hedgehog Pathway Inhibition for the Treatment of Basal Cell Carcinoma.

Gutzmer R., Solomon J.A.

Targeted Oncology. 14(3) (pp 253-267), 2019. Date of Publication: 01 Jun 2019.

[Review]

Publisher

Springer-Verlag France (22, Rue de Palestro, Paris 75002, France)

→ Excluded after screening, review

38.

Targeted biological drugs and immune check point inhibitors for locally advanced or metastatic cancers of the conjunctiva, eyelid, and orbit.

Esmaeli B., Sagiv O.

International Ophthalmology Clinics. 59(2) (pp 13-26), 2019. Date of Publication: 01 Mar 2019.

[Article]

Publisher

→ Excluded after screening, aBCC

0.

Targeting the hedgehog pathway in cancer: Current evidence and future perspectives.

Girardi D., Barrichello A., Fernandes G., Pereira A.

Cells. 8(2) (no pagination), 2019. Article Number: 153. Date of Publication: 2019.

[Review]

Publisher

MDPI AG (Postfach, Basel CH-4005, Switzerland. E-mail: indexing@mdpi.com)

➔ Excluded after screening, review

41.

Hedgehog Pathway Inhibitors and Their Utility in Basal Cell Carcinoma: A Comprehensive Review of Current Evidence.

Tay E.Y.-X., Teoh Y.-L., Yeo M.S.-W.

Dermatology and Therapy. 9(1) (pp 33-49), 2019. Date of Publication: 01 Mar 2019.

[Review]

Publisher

➔ Excluded after screening, review

42.

Nonmelanoma Skin Cancer.

Brandt M.G., Moore C.C.

Facial Plastic Surgery Clinics of North America. 27(1) (pp 1-13), 2019. Date of Publication: February 2019.

[Review]

Publisher

W.B. Saunders

→ excluded after screening, review

43.

Sonidegib in nevoid basal cell carcinoma syndrome.

Canha C., Bajric B., Martinez A.E., Rana F.

Journal of Investigative Medicine. Conference: Southern Regional Meeting 2019. New Orleans, LA United States. 67(2) (pp 433-434), 2019. Date of Publication: February 2019.

→ Excluded after eligibility assessment, sonidegib for BCNS, does not report on reoccurrence/resistance/dosing regiment/QoL

45.

A rare presentation of multiple scrotal basal cell carcinomas secondary to Gorlin's syndrome.

Rohan P., Shilling C., Shah N., Daly P., Cullen I.

Journal of Clinical Urology. (no pagination), 2019. Date of Publication: 2019.

→ Excluded after eligibility assessment, does not discuss HPI treatment

46.

Advanced basal cell cancer: Concise review of molecular characteristics and novel targeted and immune therapeutics.

Nikanjam M., Cohen P.R., Kato S., Sicklick J.K., Kurzrock R.

Annals of Oncology. 29(11) (pp 2192-2199), 2018. Date of Publication: November 2018.

[Review]

Publisher

Oxford University Press

→ Excluded after screening, review

47.

Current trends in Hedgehog signaling pathway inhibition by small molecules.

Ghirga F., Mori M., Infante P.

Bioorganic and Medicinal Chemistry Letters. 28(19) (pp 3131-3140), 2018. Date of Publication: 15 October 2018.

[Review]

Publisher

Elsevier Ltd

→ Excluded after screening, review

49.

Programmed death-1 blockade for multiple basal cell carcinomas: clearing the field systemically?.

Haug V., Schilling B.

British Journal of Dermatology. 179(3) (pp 566-567), 2018. Date of Publication: September 2018.

[Note]

Publisher

Blackwell Publishing Ltd

→ Excluded after screening, different treatment

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Effective anti-programmed death-1 therapy in a SUFU-mutated patient with Gorlin-Goltz syndrome.

Moreira A., Kirchberger M.C., Toussaint F., Erdmann M., Schuler G., Heinzerling L.

British Journal of Dermatology. 179(3) (pp 747-749), 2018. Date of Publication: September 2018.

[Article]

Publisher

Blackwell Publishing Ltd

→ Excluded after screening, different treatment

52.

SOX9 Transcriptionally Regulates mTOR-Induced Proliferation of Basal Cell Carcinomas.

Kim A.L., Back J.H., Chaudhary S.C., Zhu Y., Athar M., Bickers D.R.

Journal of Investigative Dermatology. 138(8) (pp 1716-1725), 2018. Date of Publication: August 2018.

[Article]

Publisher

Elsevier B.V.

→ Excluded after screening, cell research

53.

Illuminating Alternative Strategies to Treat Targeted Chemotherapy-Resistant Sporadic Basal Cell Carcinoma.

Nguyen T.T.L., Atwood S.X.

Journal of Investigative Dermatology. 138(5) (pp 1017-1019), 2018. Date of Publication: May 2018.

→ Excluded after screening, commentary

54.

Vismodegib-resistant basal cell carcinomas in basal cell nevus syndrome: Clinical approach and genetic analysis.

Sinx K.A.E., Roemen G.M.J.M., van Zutven V., Janssen R., Speel E.-J.M., Steijlen P.M., van Geel M., Mosterd K.

JAAD Case Reports. 4(5) (pp 408-411), 2018. Date of Publication: June 2018.

→ Included after eligibility assessment, vismdoegib for BCNS discusses reoccurrence

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Novel patched 1 mutations in patients with gorlin-goltz syndrome strategic treated by smoothed inhibitor.

Hsu S.-W., Lin C.-Y., Wang C.-W., Chung W.-H., Yang C.-H., Chang Y.-Y.

Annals of Dermatology. 30(5) (pp 597-601), 2018. Date of Publication: October 2018.

➔ Excluded after eligibility assessment, vismodegib for BCNS family, does not report on reoccurrence/resistance/dosing regiment/QoL

56.

Advances in genetic understanding of gorlin syndrome and emerging treatment options.

Shih S., Dai C., Ansari A., Urso B.A., Laughlin A.I., Solomon J.A.

Expert Opinion on Orphan Drugs. 6(7) (pp 413-423), 2018. Date of Publication: 03 Jul 2018.

[Review]

Publisher

Taylor and Francis Ltd (E-mail: [healthcare.enquiries@informa.com](mailto:healthcare.enquiries@informa.com))

➔ Excluded after screening, review

57.

Gorlin Syndrome. Sindrome de Gorlin <Sindrome de Gorlin.>

Palacios-Alvarez I., Gonzalez-Sarmiento R., Fernandez-Lopez E.

Actas Dermo-Sifiliograficas. 109(3) (pp 207-217), 2018. Date of Publication: April 2018.

➔ Excluded after screening, review

59.

Basal cell nevus syndrome (Gorlin syndrome): genetic insights, diagnostic challenges, and unmet milestones.

Akbari M., Chen H., Guo G., Legan Z., Ghali G.

Pathophysiology. 25(2) (pp 77-82), 2018. Date of Publication: June 2018.

➔ Excluded after eligibility assessment, does not report on treatment with HPis

60.

Mosaic SMO mutation in a patient with linear basal follicular hamartomas confirms Happle-Tinschert syndrome as a variant of Curry-Jones syndrome.

Lovgren M.-L., Twigg S.R.F., Moss C.

British Journal of Dermatology. Conference: 98th Annual Meeting of the British Association of Dermatologists, BAD 2018. Edinburgh United Kingdom. 179(Supplement 1) (pp 78-79), 2018. Date of Publication: July 2018.

➔ Excluded after eligibility assessment, does not report on treatment with HPis

61.

Bromodomain-containing proteins BRD7 and BRD9 are novel interacting regulators of BCC resistance.

Kim A.L., Jin G.C., Zhu Y., Bickers D.R.

Journal of Investigative Dermatology. Conference: 2018 Annual Meeting of the International Investigative Dermatology, IID 2018. Orlando, FL United States. 138(5 Supplement 1) (pp S38), 2018. Date of Publication: May 2018.

➔ Excluded after screening, markers for resistance to vismodegib

62.

Confocal microscopy in management of gorlin syndrome.

Zaman S., Ravenscroft J., Flohr C., Craythorne E.

Pediatric Dermatology. Conference: 18th Annual Meeting of the European Society for Pediatric Dermatology, ESPD 2018. London United Kingdom. 35(Supplement 2) (pp S20), 2018. Date of Publication: April 2018.

➔ Excluded after screening, RCM for management of BCCs in gorlin children

63.

Case report: Use of vismodegib in a patient with Gorlin Goltz syndrome.

Gonzalez Gonzalez M.A., Ferreras N., Matilla B., Martinez E., Nieto B., Ruano R.

European Journal of Hospital Pharmacy. Conference: 23rd Congress of the European Association of Hospital Pharmacists, EAHP 2018. Gothenburg Sweden. 25(Supplement 1) (pp A97-A98), 2018. Date of Publication: March 2018.

→ Duplicate

66.

What's new with common genetic skin disorders?.

Ma J.E., Hand J.L.

Minerva Pediatrica. 69(4) (pp 288-297), 2017. Date of Publication: August 2017.

→ Excluded after screening, review

67.

Spectrum of orocutaneous disease associations: Genodermatoses and inflammatory conditions.

Wilder E.G., Frieder J., Sulhan S., Michel P., Cizenski J.D., Wright J.M., Menter M.A.

Journal of the American Academy of Dermatology. 77(5) (pp 809-830), 2017. Date of Publication: November 2017.

Mosby Inc. (E-mail: customerservice@mosby.com)

→ Excluded after screening, review

70.

Molecular basis of basal cell carcinoma.

Montagna E., Lopes O.S.

Anais Brasileiros de Dermatologia. 92(4) (pp 517-520), 2017. Date of Publication: July-August 2017.



➔ Excluded after screening, review

71.

Nevoid basal cell carcinoma syndrome (gorlin-goltz syndrome): A patient showing distinctive dermoscopic features: Case report.

Yorulmaz A., Atilan A.U., Yalcin B.

Turkiye Klinikleri Dermatoloji. 27(2) (pp 80-85), 2017. Date of Publication: 2017.

[Article]

Publisher

OrtadogAYu Reklam Tanitim Yayıncılık Turizm Egitim Insaat Sanayi ve Ticaret A.S. (Turkocagi Caddesi No. 30, Balgat 06520, Turkey. E-mail: [aysea@turkiyeklinikleri.com](mailto:aysea@turkiyeklinikleri.com))

➔ Excluded after screening, not treated with hedgehog pathway inhibitor

72.

18th Annual Scientific Meeting of the NVED 2017.

Anonymous

Nederlands Tijdschrift voor Dermatologie en Venereologie. Conference: 18th Annual Scientific Meeting of the Nederlandse Vereniging voor Experimentele Dermatologie, NVED 2017. Lunteren Netherlands. 27(1) (no pagination), 2017. Date of Publication: January 2017.

[Conference Review]

Publisher

DCHG Partners in Mediscne Communicatie

➔ Excluded after screening, no information

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Predicting response to vismodegib before treatment initiation based on baseline characteristics in patients with metastatic basal cell carcinoma in a pooled analysis.

Grob J.-J., Hansson J., Basset-Seguín N., Kunstfeld R., Dreno B., Mortier L., Ascierto P., Licitra L., Hertig C., Dimier N., Fittipaldo A., Hauschild A.

Journal of the European Academy of Dermatology and Venereology. Conference: 13th Congress of the European Association of Dermato-Oncology, EADO 2017. Athens Greece. 31(Supplement 3) (pp 80-81), 2017. Date of Publication: June 2017.

[Conference Abstract]

Publisher

Blackwell Publishing Ltd

➔ Excluded after screening, research on mBCC

➔

80.

Vismodegib treatment in a multidisciplinary team, Aarhus Denmark - One year intention-to-treat cohort analysis and recommendations.

Lorentzen H.F., Heje M., Als A.B.

Journal of the European Academy of Dermatology and Venereology. Conference: 13th Congress of the European Association of Dermato-Oncology, EADO 2017. Athens Greece. 31(Supplement 3) (pp 88), 2017. Date of Publication: June 2017.

[Conference Abstract]

➔ Excluded after eligibility assessment, does not discuss outcomes for multiple BCCs

82.

Abstracts of the 13th Congress of the European Association of Dermato-Oncology, EADO 2017.

Anonymous

Journal of the European Academy of Dermatology and Venereology. Conference: 13th Congress of the European Association of Dermato-Oncology, EADO 2017. Athens Greece. 31(Supplement 3) (no pagination), 2017. Date of Publication: June 2017.

[Conference Review]

Publisher

Blackwell Publishing Ltd

→ Excluded after screening, no information

85.

Basal cell nevus syndrome (Gorlin-Goltz syndrome): Genetic predisposition, clinical picture and treatment.

Witmanowski H., Szychta P., Blochowiak K., Jundzill A., Czajkowski R.

Postepy Dermatologii i Alergologii. 34(4) (pp 381-387), 2017. Date of Publication: August 2017.

[Letter]

Publisher

Termedia Publishing House Ltd. (Kleeberqa St.2, Poznan 61-615, Poland)

→ Excluded after screening, small review

86.

Implementation of systemic hedgehog inhibitors in daily practice as neoadjuvant therapy.

Tang N., Ratner D.

JNCCN Journal of the National Comprehensive Cancer Network. 15(4) (pp 537-543), 2017. Date of Publication: 01 Apr 2017.

[Review]

Publisher

Harborside Press (37 main Street, Cold Spring Harbor NY 11724, United States)

→ Excluded after screening, review

88.

Ocular basal cell carcinoma: A brief literature review of clinical diagnosis and treatment.

Shi Y., Jia R., Fan X.

OncoTargets and Therapy. 10 (pp 2483-2489), 2017. Date of Publication: 08 May 2017.

[Article]

Publisher

Dove Medical Press Ltd. (PO Box 300-008, Albany, Auckland, New Zealand)

➔ Excluded after screening, review

90.

Segmental basal cell naevus syndrome caused by an activating mutation in smoothed.

Khamaysi Z., Bochner R., Indelman M., Magal L., Avitan-Hersh E., Sarig O., Sprecher E., Bergman R.

British Journal of Dermatology. (no pagination), 2016. Date of Publication: 2016.

[Article In Press]

Publisher

Blackwell Publishing Ltd (E-mail: [customerservices@oxonblackwellpublishing.com](mailto:customerservices@oxonblackwellpublishing.com))

➔ Excluded after screening, no treatment

91.

Hijacking the hedgehog pathway in cancer therapy.

Laukkanen M.O., Castellone M.D.

Anti-Cancer Agents in Medicinal Chemistry. 16(3) (pp 309-317), 2016. Date of Publication: 01 Mar 2016.

[Article]

Publisher

Bentham Science Publishers (P.O. Box 294, Bussum 1400 AG, Netherlands)

➔ Excluded after screening, review

92.

Hedgehog blockade for basal cell carcinoma coming at a (secondary neoplastic) price.

Rubben A., Hilgers R.-D., Leverkus M.

JAMA Dermatology. 152(5) (pp 521-523), 2016. Date of Publication: May 2016.

[Review]

Publisher

American Medical Association (E-mail: [smcleod@itsa.ucsf.edu](mailto:smcleod@itsa.ucsf.edu))

→ Excluded after screening, review

96.

Recent trends in the treatment of benign odontogenic tumors.

Covello P., Buchbinder D.

Current Opinion in Otolaryngology and Head and Neck Surgery. 24(4) (pp 343-351), 2016. Date of Publication: 01 Aug 2016.

[Review]

Publisher

Lippincott Williams and Wilkins (E-mail: [kathiest.clai@apta.org](mailto:kathiest.clai@apta.org))-

→ Excluded after screening, review on odontogenic keratocysts

97.

AKT1 activation is obligatory for spontaneous BCC tumor growth in a murine model that mimics some features of basal cell nevus syndrome.

Kim A.L., Back J.H., Zhu Y., Tang X., Yardley N.P., Kim K.J., Athar M., Bickers D.R.

Cancer Prevention Research. 9(10) (pp 794-802), 2016. Date of Publication: October 2016.

[Article]

Publisher

American Association for Cancer Research Inc. (E-mail: [helen.atkins@aacr.org](mailto:helen.atkins@aacr.org))

→ Excluded after screening, molecular/cell research

98.

Safety and efficacy of vismodegib in patients with basal cell carcinoma nevus syndrome: Pooled analysis of two trials.

Chang A.L.S., Arron S.T., Migden M.R., Solomon J.A., Yoo S., Day B.-M., McKenna E.F., Sekulic A.

Orphanet Journal of Rare Diseases. 11(1) (no pagination), 2016. Article Number: 120. Date of Publication: 01 Sep 2016.

➔ Excluded after eligibility assessment, only results for aBCC

103.

Can hair re-growth be considered an early clinical marker of treatment resistance to Hedgehog inhibitors in patients with advanced basal cell carcinoma? A report of two cases.

Soura E., Plaka M., Dessinioti C., Syrigos K., Stratigos A.J.

Journal of the European Academy of Dermatology and Venereology. 30(10) (pp 1726-1729), 2016. Date of Publication: 01 Oct 2016.

➔ Included after eligibility assessment, resistance and reoccurrence in BCCs BCNS patient with vismodegib

107.

Familial skin cancer syndromes Increased risk of nonmelanotic skin cancers and extracutaneous tumors.

Jaju P.D., Ransohoff K.J., Tang J.Y., Sarin K.Y.

Journal of the American Academy of Dermatology. 74(3) (pp 437-451), 2016. Date of Publication: 01 Mar 2016.

➔ Excluded after screening, review

109.

Basal cell naevus syndrome: An update on genetics and treatment.

John A.M., Schwartz R.A.

British Journal of Dermatology. 174(1) (pp 68-76), 2016. Date of Publication: 01 Jan 2016.

[Article]

Publisher

Blackwell Publishing Ltd (E-mail: [customerservices@oxonblackwellpublishing.com](mailto:customerservices@oxonblackwellpublishing.com))

→ Excluded after screening, guideline

110.

The burden of illness in patients with basal cell nevus syndrome: United States patient registry experience.

Solis D.C., Kwon G., Ransohoff K.J., Chahal H.S., Lindgren J.A., Li S., Ally M.S., Peters M., Teng J., Burr K., Epstein E., Tang J.

Journal of Investigative Dermatology. Conference: 2016 Annual Meeting of the Society for Investigative Dermatology, SID 2016. Scottsdale, AZ United States. 136(8) (pp B5), 2016. Date of Publication: August 2016.

→ Excluded after eligibility assessment, does not report outcome of QoL during hedgehog pathway inhibitor treatment

113.

Novel SUFU splice mutation in a child with multiple tumors.

Walsh M.F., Harlan M., Kennedy J., Musinsky J., LaQuaglia M., Stadler Z., Gilheaney S., Offit K.

Cancer Research. Conference: AACR Special Conference on Advances in Pediatric Cancer Research: From Mechanisms and Models to Treatment and Survivorship 2015. Fort Lauderdale, FL United States. 76(5 Supplement) (no pagination), 2016. Date of Publication: March 2016.

[Conference Abstract]

Publisher

American Association for Cancer Research Inc.

→ Excluded after screening, not treated with hedgehog pathway inhibitor

115.

Long-term follow-up of two patients with Nevoid basal cell carcinoma syndrome (NBCCS) treated with Vismodegib.

Van Eecke L., Wolter P., Bechter O., Rogiers A., De Smedt J., Garmyn M.

Melanoma Research. Conference: 16th World Congress on Cancers of the Skin 2016. Vienna Austria. 26(Supplement 1) (pp e75-e76), 2016. Date of Publication: August 2016.

[Conference Abstract]

→ included after eligibility assesment, long term vismodegib in bcns with reoccurence

## Long-term Response to Vismodegib in a Patient with Gorlin-Goltz Syndrome: A Case Report and Review of Pathological Mechanisms Involved

Meghana Kesireddy , Vincent L. Mendiola , Bagi Jana , Shrestha Patel

→ Included after eligibility assesment

117.

Gorlin syndrome with locally advanced basal cell carcinomas treated with vismodegib.

Abeldano A., Maskin M., Arias M., Gonzalez A., Benedetti A., Lamas C.

Melanoma Research. Conference: 16th World Congress on Cancers of the Skin 2016. Vienna Austria. 26(Supplement 1) (pp e88), 2016. Date of Publication: August 2016.

[Conference Abstract]

Publisher

Lippincott Williams and Wilkins

→ Excluded after screening, laBCC

119.

Resistances to vismodegibs in a French case series of 207 patients with locally advanced basal cell carcinoma.

Basset-Seguín N., Sharpe H., Poulalhon N., Mortier L., Saiag P., Monestier S., Hou J., Bagot M., Guillot B., Robert C., Meyer N., Khammari A., Grange F., Dutriaux C., Dreno B., De Sauvage F.

Journal of Clinical Oncology. Conference: 2016 Annual Meeting of the American Society of Clinical Oncology, ASCO 2016. Chicago, IL United States. 34(Supplement 15) (no pagination), 2016. Date of Publication: May 2016.

[Conference Abstract]

Publisher

American Society of Clinical Oncology

→ Excluded after screening, resistance in laBCC



122.

Ingenol Mebutate Treatment in a Patient with Gorlin Syndrome.

Stieger M., Hunger R.E.

Dermatology. 232(1 Supplement 1) (pp 29-31), 2016. Date of Publication: 01 Aug 2016.

[Article]

Publisher

S. Karger AG

→ Excluded after screening, different treatment

125.

Non-melanoma skin cancer - Overview.

Joseph K.

Current Cancer Therapy Reviews. 12(3) (pp 142-151), 2016. Date of Publication: 01 Sep 2016.

[Review]

Publisher

Bentham Science Publishers B.V. (P.O. Box 294, Bussum 1400 AG, Netherlands)

→ Excluded after screening, review

126.

Diagnosis and management of hereditary basal cell skin cancer.

Shanley S., McCormack C.

Recent Results in Cancer Research. 205 (pp 191-212), 2016. Date of Publication: 2016.

[Article]

Publisher

Springer New York LLC (E-mail: [barbara.b.bertram@gsk.com](mailto:barbara.b.bertram@gsk.com))

→ Excluded after screening, review/guideline

127.

Not the usual suspect: a case of basal cell naevus syndrome caused by a SMO mutation alone.

Ahn R.S.

British Journal of Dermatology. 175(1) (pp 21-22), 2016. Date of Publication: 01 Jul 2016.

[Note]

Publisher

Blackwell Publishing Ltd (E-mail: [customerservices@oxonblackwellpublishing.com](mailto:customerservices@oxonblackwellpublishing.com))

➔ Excluded after screening, no treatment with hedgehog pathway inhibitor

129.

Shh and p50/Bcl3 signaling crosstalk drives pathogenesis of BCCs in Gorlin syndrome.

Chaudhary S.C., Tang X., Arumugam A., Li C., Srivastava R.K., Weng Z., Xu J., Zhang X., Kim A.L., McKay K., Elmets C.A., Kopelovich L., Bickers D.R., Athar M.

Oncotarget. 6(34) (pp 36789-36814), 2015. Date of Publication: 2015.

[Article]

Publisher

Impact Journals LLC (E-mail: [editors@impactaging.com](mailto:editors@impactaging.com))

➔ Excluded after screening, cell/molecular research

130.

Review of ocular manifestations of nevoid basal cell carcinoma syndrome: What an ophthalmologist needs to know.

Chen J.J., Sartori J., Aakalu V.K., Setabutr P.

Middle East African Journal of Ophthalmology. 22(4) (pp 421-427), 2015. Date of Publication: October-December 2015.

➔ Excluded after screening, review

131.

Usefulness of photodynamic therapy as a possible therapeutic alternative in the treatment of basal cell carcinoma.

Savoia P., Deboli T., Previgliano A., Broganelli P.

International Journal of Molecular Sciences. 16(10) (pp 23300-23317), 2015. Date of Publication: 28 Sep 2015.

→ Excluded after screening, review

134.

Paediatric dermatology highlights.

Dinulos J.G.

Current Opinion in Pediatrics. 27(4) (pp 453), 2015. Date of Publication: 01 Aug 2015.

→ Excluded after screening, review

139.

Targeting Notch, Hedgehog, and Wnt pathways in cancer stem cells: Clinical update.

Takebe N., Miele L., Harris P.J., Jeong W., Bando H., Kahn M., Yang S.X., Ivy S.P.

Nature Reviews Clinical Oncology. 12(8) (pp 445-464), 2015. Date of Publication: 30 Aug 2015.

[Review]

Publisher

Nature Publishing Group (Houndmills, Basingstoke, Hampshire RG21 6XS, United Kingdom)

→ Excluded after screening, review

140.

Hedgehog signaling and urological cancers.

Shigemura K., Fujisawa M.

Current Drug Targets. 16(3) (pp 258-271), 2015. Date of Publication: 2015.

[Article]

Publisher

Bentham Science Publishers B.V. (P.O. Box 294, Bussum 1400 AG, Netherlands)

→ Excluded after screening, different disease

141.

Gorlin syndrome and desmoplastic medulloblastoma: Report of 3 cases with unfavorable clinical course and novel mutations.

Gururangan S., Robinson G., Ellison D.W., Wu G., He X., Lu Q.R., McLendon R., Grant G., Driscoll T., Neuberg R.

Pediatric Blood and Cancer. 62(10) (pp 1855-1858), 2015. Date of Publication: 01 Oct 2015.

➔ Excluded after screening, different gorlin symptom

142.

Characteristics and outcomes of nonmelanoma skin cancer (NMSC) in children and young adults.

Khosravi H., Schmidt B., Huang J.T.

Journal of the American Academy of Dermatology. 73(5) (pp 785-790), 2015. Date of Publication: November 2015.

[Article]

Publisher

Mosby Inc. (E-mail: [customerservice@mosby.com](mailto:customerservice@mosby.com))

➔ Excluded after eligibility assessment, only 1 patient received vismodegib and nothing is mentioned on outcome

144.

Advanced basal cell carcinoma, the hedgehog pathway, and treatment options - Role of smoothed inhibitors.

Fecher L.A., Sharfman W.H.

Biologics: Targets and Therapy. 9 (pp 129-140), 2015. Date of Publication: 06 Nov 2015.

[Review]

Publisher

Dove Medical Press Ltd. (PO Box 300-008, Albany, Auckland, New Zealand)

➔ Excluded after screening, review aBCC

145.

Digging a hole under Hedgehog: Downstream inhibition as an emerging anticancer strategy.

Di Magno L., Coni S., Di Marcotullio L., Canettieri G.

Biochimica et Biophysica Acta - Reviews on Cancer. 1856(1) (pp 62-72), 2015. Date of Publication: August 01, 2015.

➔ Excluded after screening, review

148.

Cooperative integration between HEDGEHOG-GLI signalling and other oncogenic pathways: Implications for cancer therapy.

Pandolfi S., Stecca B.

Expert Reviews in Molecular Medicine. 17 (no pagination), 2015. Article Number: e5. Date of Publication: 30 Jan 2015.

➔ Excluded after screening, review

150.

Basal cell carcinoma: Clinical practice assessment and educational gap analysis.

Herrmann T., Pearce F., Williamson C., Peters P., Weiss G.J.

Journal of the American Academy of Dermatology. Conference: 73rd Annual Meeting of the American Academy of Dermatology. San Francisco, CA United States. Conference Publication: (var.pagings). 72(5 SUPPL. 1) (pp AB181), 2015. Date of Publication: May 2015.

➔ Excluded after screening, lecture

152.

Smoothed (SMO) resistance is driven by PI3K-Akt signaling in a subset of murine ASZ001 BCC cells displaying tumor-initiating cell (TIC)-like characteristics.

Jin G.C., Zhu Y., Kim A., Bickers D.R.

Journal of Investigative Dermatology. Conference: 2015 Annual Meeting of the Society for Investigative Dermatology. Atlanta, GA United States. Conference Publication: (var.pagings). 135(SUPPL. 1) (pp S20), 2015. Date of Publication: May 2015.

➔ Excluded after screening, cell/molecular research

158.

Gorlin syndrome (nevoid basal cell carcinoma syndrome): Update and literature review.

Fujii K., Miyashita T.

Pediatrics International. 56(5) (pp 667-674), 2014. Date of Publication: 01 Oct 2014.

➔ Excluded after screening (review)

163.

The spectrum of oculocutaneous disease: Part II. Neoplastic and drug-related causes of oculocutaneous disease.

Day A., Abramson A.K., Patel M., Warren R.B., Menter M.A.

Journal of the American Academy of Dermatology. 70(5) (pp e1-821), 2014. Date of Publication: 2014.

[Article]

Publisher

Mosby Inc. (E-mail: [customerservice@mosby.com](mailto:customerservice@mosby.com))

➔ Excluded after screening, aBCC

165.

Targeted therapy for hereditary cancer syndromes: Neurofibromatosis type 1, neurofibromatosis type 2, and gorlin syndrome.

Agarwal R., Liebe S., Turski M.L., Vidwans S.J., Janku F., Garrido-Laguna I., Munoz J., Schwab R., Rodon J., Kurzrock R., Subbiah V.

Discovery Medicine. 18(101) (pp 323-330), 2014. Date of Publication: 2014.

➔ Excluded after screening, review

167.

Case: Targeted therapy-vismodegib in the management of recurrent basal cell carcinoma in gorlin syndrome.

Kelly W., Kelly C., Hanrahan E.O., Ballot J.

Irish Journal of Medical Science. Conference: RAMI Intern Section Meeting 2014. Dublin Ireland.  
Conference Publication: (var.pagings). 183(4 SUPPL. 1) (pp S145), 2014. Date of Publication: July 2014.

➔ Excluded after eligibility assessment, does not report outcome in the abstract

170.

Interaction of hedgehog and vitamin D signaling pathways in basal cell carcinomas.

Albert B., Hahn H.

Advances in Experimental Medicine and Biology. 810 (pp 329-341), 2014. Date of Publication: 2014.

➔ Excluded after screening, book chapter

171.

Targeted therapy for advanced basal-cell carcinoma: Vismodegib and beyond.

Cowey C.L.

Dermatology and Therapy. 3(1) (pp 17-31), 2013. Date of Publication: June 2013.

➔ Excluded after screening, review

173.

Inhibition of hedgehog/Gli signaling by botanicals: A review of compounds with potential hedgehog pathway inhibitory activities.

Drenkhahn S.K., Jackson G.A., Slusarz A., Starkey N.J.E., Lubahn D.B.

Current Cancer Drug Targets. 13(5) (pp 580-595), 2013. Date of Publication: 2013.

➔ Excluded after screening, review of compounds with potential hedgehog pathway inhibition

177.

New prospects for drug development: The hedgehog pathway revealed. Focus on hematologic malignancies.

Pimentel A., Velez M., Barahona L.J., Swords R., Lekakis L.

Future Oncology. 9(5) (pp 681-697), 2013. Date of Publication: May 2013.

➔ Excluded after screening, review

178.

Systemic treatment for hereditary cancers: A 2012 update.

Imyanitov E.N., Byrski T.

Hereditary Cancer in Clinical Practice. 11(1) (no pagination), 2013. Article Number: 2. Date of Publication: 01 Apr 2013.

[Review]

Publisher

BioMed Central Ltd. (Floor 6, 236 Gray's Inn Road, London WC1X 8HB, United Kingdom)

➔ Excluded after screening, review

179.

Systemic therapy for inoperable and metastatic basal cell cancer.

Fecher L.A.

Current Treatment Options in Oncology. 14(2) (pp 237-248), 2013. Date of Publication: June 2013.

[Article]

Publisher

Springer New York (233 Spring Street, New York NY 10013-1578, United States)

➔ Excluded after screening, review

180.

Brief S2k guidelines - Basal cell carcinoma of the skin. S2k Kurzleitlinie - Basalzellkarzinom der Haut <S2k Kurzleitlinie - Basalzellkarzinom der Haut.>

Hauschild A., Breuninger H., Kaufmann R., -DieterKortmann R., Klein M., Werner J., Reifenberger J., Dirschka T., Garbe C.

JDDG - Journal of the German Society of Dermatology. 11(SUPPL. 3) (pp 10-15), 2013. Date of Publication: June 2013.

[Article]

Publisher



Blackwell Publishing Ltd (9600 Garsington Road, Oxford OX4 2XG, United Kingdom)

➔ Excluded after screening, guidelines BCC

181.

The Hedgehog signalling pathway in breast development, carcinogenesis and cancer therapy.

Hui M., Cazet A., Nair R., Watkins D.N., O'Toole S.A., Swarbrick A.

Breast Cancer Research. 15(2) (no pagination), 2013. Article Number: 203. Date of Publication: 28 Mar 2013.

[Review]

Publisher

BioMed Central Ltd. (Floor 6, 236 Gray's Inn Road, London WC1X 8HB, United Kingdom)

➔ Excluded after screening, review on different disease

182.

Therapeutic targeting of developmental signaling pathways in medulloblastoma: Hedgehog, Notch, Wnt and Myc.

Raabe E., Eberhart C.G.

Current Signal Transduction Therapy. 8(1) (pp 55-66), 2013. Date of Publication: April 2013.

[Article]

Publisher

Bentham Science Publishers B.V. (P.O. Box 294, Bussum 1400 AG, Netherlands)

➔ Excluded after screening, different disease

184.

Targeting hedgehog signaling in cancer: Research and clinical developments.

Xie J., Bartels C.M., Barton S.W., Gu D.

OncoTargets and Therapy. 6 (pp 1425-1435), 2013. Date of Publication: 2013.

[Review]

Publisher

Dove Medical Press Ltd. (PO Box 300-008, Albany, Auckland, New Zealand)

→ Excluded after screening, review

186.

Cancer-associated genodermatoses: Skin neoplasms as clues to hereditary tumor syndromes.

Ponti G., Pellacani G., Seidenari S., Pollio A., Muscatello U., Tomasi A.

Critical Reviews in Oncology/Hematology. 85(3) (pp 239-256), 2013. Date of Publication: March 2013.

[Review]

Publisher

Elsevier Ireland Ltd (P.O. Box 85, Limerick, Ireland)

→ Excluded after screening, review

188.

Comment on basal cell carcinoma rebound after cessation of vismodegib in an individual with basal cell nevus syndrome.

Ally M.S., Wysong A., Tang J.Y., Aasi S.

Dermatologic Surgery. 39(9) (pp 1413-1414), 2013. Date of Publication: September 2013.

[Letter]

Publisher

Blackwell Publishing Ltd (9600 Garsington Road, Oxford OX4 2XG, United Kingdom)

→ Excluded after eligibility assessment, discusses no new cases

189.

Role of the Hedgehog pathway in hepatocellular carcinoma (Review).

Zheng X., Zeng W., Gai X., Xu Q., Li C., Liang Z., Tuo H., Liu Q.

Oncology Reports. 30(5) (pp 2020-2026), 2013. Date of Publication: November 2013.

[Review]

Publisher

Spandidos Publications Ltd. (10 Vriaxidos Street, Athens 11635, Greece)

➔ Excluded after screening, review different disease

190.

Targeted therapies and basal cell carcinoma.

Basset-Seguin N.

Oncologie. 15(2) (pp 101-105), 2013. Date of Publication: January 2013.

[Review]

Publisher

Springer Paris (1 rue Paul Cezanne, Paris 75008, France)

➔ Excluded after screening, review

197.

A better way forward: Targeting hedgehog signaling in liver cancer.

Kappler R., Von Schweinitz D.

Frontiers in Bioscience - Scholar. 4 S(1) (pp 277-286), 2012. Date of Publication: 01 Jan 2012.

[Article]

Publisher

Frontiers in Bioscience

➔ Excluded after screening, different disease

198.

Basal cell carcinoma: Topical therapy versus surgical treatment.

Sharquie K.E., Noaimi A.A.

Journal of the Saudi Society of Dermatology and Dermatologic Surgery. 16(2) (pp 41-51), 2012. Date of Publication: July 2012.

[Review]

Publisher

Elsevier

➔ Excluded after screening, review

200.

Targeting the Hedgehog signaling pathway for cancer therapy.

Li Y., Maitah M.Y., Ahmad A., Kong D., Bao B., Sarkar F.H.

Expert Opinion on Therapeutic Targets. 16(1) (pp 49-66), 2012. Date of Publication: January 2012.

[Review]

Publisher

Informa Healthcare (69-77 Paul Street, London EC2A 4LQ, United Kingdom)

➔ Excluded after screening, review

201.

Adult medulloblastoma, from spongioblastoma cerebelli to the present day: A review of treatment and the integration of molecular markers.

Shonka N., Brandes A., de Groot J.F.

ONCOLOGY (United States). 26(11) (no pagination), 2012. Date of Publication: 2012.

[Review]

Publisher

UBM Medica Healthcare Publications (PO Box 390427, Minneapolis MN 55439, United States)

➔ Excluded after screening, different disease

202.

New systemic treatment options for advanced basal cell carcinoma.

Guminski A.

Cancer Forum. 36(3) (no pagination), 2012. Date of Publication: November 2012.

[Review]

Publisher

Cancer Council Australia (Level 1, 120 chambers street, Surry Hills NSW 2010, Australia)

➔ Excluded after screening, review

203.

Urticaria after methyl aminolevulinate photodynamic therapy in a patient with nevoid basal cell carcinoma syndrome.

Wolfe C.M., Green W.H., Hatfield H.K., Cognetta Jr. A.B.

Journal of Drugs in Dermatology. 11(11) (pp 1364-1365), 2012. Date of Publication: November 2012.

[Article]

Publisher

Journal of Drugs in Dermatology (377 Park Avenue South, New York NY 10016, United States)

➔ Excluded after screening, different treatment

204.

Targeting hedgehog in hematologic malignancy.

Irvine D.A., Copland M.

Blood. 119(10) (pp 2196-2204), 2012. Date of Publication: 08 Mar 2012.

[Review]

Publisher

American Society of Hematology (1900 M Street, Suite 2000, Washington DC 20036, United

→ excluded after screening, different disease

205.

Investigational agents in metastatic basal cell carcinoma: Focus on vismodegib.

Batty N., Kossoff E., Dy G.K.

Journal of Experimental Pharmacology. 4(1) (pp 97-103), 2012. Date of Publication: 2012.

[Review]

Publisher

DOVE Medical Press Ltd. (PO Box 300-008, Albany, Auckland, New Zealand)

→ Excluded after screening, review

207.

Advances in targeting the Hedgehog signaling pathway in cancer therapy.

Kiesslich T., Neureiter D.

Expert Opinion on Therapeutic Targets. 16(2) (pp 151-156), 2012. Date of Publication: February 2012.

[Review]

Publisher

Informa Healthcare (69-77 Paul Street, London EC2A 4LQ, United Kingdom)

→ Excluded after screening, review

209.

Metastatic basal cell carcinoma in the era of hedgehog signaling pathway inhibitors.

Weiss G.J., Korn R.L.

Cancer. 118(21) (pp 5310-5319), 2012. Date of Publication: 01 Nov 2012.

[Review]

Publisher

John Wiley and Sons Inc. (P.O.Box 18667, Newark NJ 07191-8667, United States)

→ Excluded after screening, review

210.

Introduction: Advanced cutaneous malignancies.

Glass L.F., Deconti R.C., Sondak V.K.

Seminars in Oncology. 39(2) (pp 132-133), 2012. Date of Publication: April 2012.

[Editorial]

Publisher

W.B. Saunders (Independence Square West, Philadelphia PA 19106-3399, United States)

→ excluded after screening, editorial

213.

The hedgehog signaling pathway in basal cell carcinoma-from bench to bedside.

Caro I.

Wound Repair and Regeneration. Conference: Conjoint 3rd Australasian Wound and Tissue Repair Society and 9th Australasian Society for Dermatology Research Conference. Sydney Australia. Conference Publication: (var.pagings). 20(5) (pp A55), 2012. Date of Publication: September-October 2012.

[Conference Abstract]

Publisher

Blackwell Publishing Inc.

→ Excluded after screening, no study in patients

215.

The oral hedgehog inhibitor vismodegib (GDC-0449) in the treatment of locally advanced basal cell carcinoma: Experience of one Australian centre.

Intong L.R.A., Rhodes L.M., Caro I., Murrell D.F.

Australasian Journal of Dermatology. Conference: 45th Annual Scientific Meeting of the Australasian College of Dermatologists. Brisbane, QLD Australia. Conference Publication: (var.pagings). 53(SUPPL. 1) (pp 40), 2012. Date of Publication: May 2012.

[Conference Abstract]

→ Excluded after screening, treatment of laBCC

216.

Hedgehog pathway as a drug target: Smoothened inhibitors in development.

Lin T.L., Matsui W.

OncoTargets and Therapy. 5 (pp 47-58), 2012. Date of Publication: 2012.

[Review]

Publisher

DOVE Medical Press Ltd. (PO Box 300-008, Albany, Auckland, New Zealand)

-> excluded after screening, review

219.

Vismodegib is effective in the treatment and prevention of BCC.

Anonymous

Cancer Discovery. 2(8) (pp 661), 2012. Date of Publication: August 2012.

[Article]

Publisher

American Association for Cancer Research Inc. (615 Chestnut Street, 17th Floor, Philadelphia PA 19106-3483, United States)

➔ Excluded after screening, no information

220.

Initial assessment of tumor regrowth after vismodegib in advanced basal cell carcinoma.

Chang A.L.S., Oro A.E.

Archives of Dermatology. 148(11) (pp 1324-1325), 2012. Date of Publication: November 2012.

[Article]

Publisher

American Medical Association (515 North State Street, Chicago IL 60654, United States)

➔ Included after eligibility assesment, discusses regrowth in multiple BCC patients

221.

Expect a miracle.

Burr K.S., Hughes P., LaRosa S.



Journal of the Dermatology Nurses' Association. 4(1) (pp 42-44), 2012. Date of Publication: January-February 2012.

[Conference Paper]

Publisher

Lippincott Williams and Wilkins (530 Walnut Street, P O Box 327, Philadelphia PA 19106-3621, United States)

➔ Excluded after screening, patient story

222.

Emerging trends and treatment approaches in nonmelanoma skin cancer: A Canadian perspective.

Kuzel P., Green J.B., Metelitsa A.I.

Journal of Cutaneous Medicine and Surgery. 15(SUPPL. 1) (pp S365-S370), 2011. Date of Publication: December 2011.

[Review]

Publisher

Decker Intellectual Properties (E-mail: [claims@sagepub.com](mailto:claims@sagepub.com))

➔ Excluded after screening, review

223.

Long-term safety, tolerability, and efficacy of vismodegib in two patients with metastatic basal cell carcinoma and basal cell nevus syndrome.

Weiss G.J., Tibes R., Blaydorn L., Jameson G., Downhour M., White E., Caro I., Von Hoff D.D.

Dermatology Reports. 3(3) (no pagination), 2011. Article Number: e55. Date of Publication: 2011.

[Article]

Publisher

- ➔ Excluded after eligibility assessment, multiple BCCs in BCNS patient, does not report on reoccurrence/resistance/dosing regiment/QoL

224.

Conference Essence in Dermatology 2011, Hong Kong.

Tang J.W.S., Lam W.Y.K., Yau E.K.Y.

Hong Kong Journal of Dermatology and Venereology. 19(4) (pp 197-201), 2011. Date of Publication: 2011.

[Conference Paper]

Publisher

Medcom Limited (18 Cheung Lee Street, Chaiwan, Hong Kong)

- ➔ Excluded after screening, conference overview

225.

Cancer interception.

Blackburn E.H.

Cancer Prevention Research. 4(6) (pp 187-192), 2011. Date of Publication: June 2011.

[Note]

Publisher

American Association for Cancer Research Inc. (615 Chestnut Street, 17th Floor, Philadelphia PA 19106-3483, United States)

- ➔ Excluded after screening, note

231.

Hereditary tumour syndromes featuring basal cell carcinomas.

Parren L.J.M.T., Frank J.

British Journal of Dermatology. 165(1) (pp 30-34), 2011. Date of Publication: July 2011.

[Review]

Publisher

Blackwell Publishing Ltd (9600 Garsington Road, Oxford OX4 2XG, United Kingdom)

→ Excluded after screening, review

233.

Whole organism based techniques and approaches in early stage oncology drug discovery-patents and trends.

Hampson R.J., Wyatt M.D.

Recent Patents on Endocrine, Metabolic and Immune Drug Discovery. 5(3) (pp 183-191), 2011. Date of Publication: September 2011.

[Article]

Publisher

Bentham Science Publishers B.V. (P.O. Box 294, Bussum 1400 AG, Netherlands)

→ Excluded after screening, drug discovery paper

234.

Scientific highlights from the 71st annual meeting of the society for investigative dermatology.

Schultz H.Y.

Journal of Investigative Dermatology. 131(10) (pp 1963-1967), 2011. Date of Publication: October 2011.

[Conference Paper]

Publisher

Nature Publishing Group (345 Park Avenue South, New York NY 10010-1707, United States)

→ Excluded after screening, review on conference

235.

Changing pathology with changing drugs: Skin cancer.

Karpova M.B., Barysch M.J., Zipser M.C., Schonewolf N., French L.E., Dummer R.

Pathobiology. 78(2) (pp 61-75), 2011. Date of Publication: June 2011.

[Review]

Publisher

S. Karger AG (Allschwilerstrasse 10, P.O. Box, Basel CH-4009, Switzerland)

→ Excluded after screening, review

236.

Vismodegib: SMO receptor antagonist hedgehog signaling inhibitor oncolytic.

Haddley K.

Drugs of the Future. 35(5) (pp 379-384), 2010. Date of Publication: May 2010.

[Article]

Publisher

Prous Science (P.O. Box 540, Barcelona 08080, Spain)

→ Excluded after screening, review

**EMBASE SONIDEGIB AND BASAL CELL CARCINOMA**

**Database: Embase <1974 to 2021 Week 37>**

**Search Strategy:**

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- 2 basal cell carcinoma/ (28620)**
- 4 basal cell nevus syndrome/ (2721)**
- 6 multiple basal cell carcinomas.mp. (472)**
- 8 sonidegib/ (873)**
- 9 2 and 8 (350)**

\*\*\*\*\*

**Duplicate: 217**

**Excluded after screening: 131**

**Eligibility assessment: 2**

**Included: 2**

17.

Rapid and exceptional response to Sonidegib in a patient with multiple locally advanced basal cell carcinomas.

Tarantino V., Zavattaro E., Veronese F., Gironi L.C., Savoia P.

Anti-Cancer Drugs. (pp 465-468), 2021. Date of Publication: 2021.

[Article]

Publisher

Lippincott Williams and Wilkins

- ➔ Excluded after eligibility assessment, case report of BCNS patient with multiple BCC treated with sonidegib, does not report on reoccurrence/resistance/dosing regiment/QoL

114.

An iatrogenic conundrum: Novel management of multiple facial BCCs years after radiotherapy for acne.

Charlton O., Phan K., Smith S.

Australasian Journal of Dermatology. Conference: 52nd Annual Scientific Meeting of the Australasian College of Dermatologists. Melbourne, VIC Australia. 60(Supplement 1) (pp 24-25), 2019. Date of Publication: May 2019.

- ➔ Excluded after eligibility assessment, case report of BCNS patient with multiple BCC treated with sonidegib, does not report on reoccurrence/resistance/dosing regiment/QoL

## EMBASE SONIDEGIB AND BASAL CELL NEVUS SYNDROME

Database: Embase <1974 to 2021 Week 37>

Search Strategy:

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1 basal cell nevus syndrome/ (2721)

2 sonidegib/ (873)

3 1 and 2 (67)

\*\*\*\*\*

Duplicates: 62

Unique: 5

Assessed: 3

Included: 2

2.

26211 Pharmacokinetics of sonidegib in patients with nevoid basal cell carcinoma syndrome.

Lear J., Hauschild A., Squittieri N., Basset-Seguin N., Dummer R.

Journal of the American Academy of Dermatology. Conference: 2021 AAD VMX Virtual Meeting. Virtual, Online. 85(3 Supplement) (pp AB90), 2021. Date of Publication: September 2021.

[Conference Abstract]

Publisher

Mosby Inc.

➔ Excluded after eligibility assessment, only discusses pharmacokinetics

3.

26206 Effect of sonidegib on secondary tumor size endpoints through 12 weeks of treatment in patients with nevoid basal cell carcinoma syndrome.

Lear J., Hauschild A., Squittieri N., Basset-Seguin N., Dummer R.

Journal of the American Academy of Dermatology. Conference: 2021 AAD VMX Virtual Meeting. Virtual, Online. 85(3 Supplement) (pp AB90), 2021. Date of Publication: September 2021.

➔ Included after eligibility assessment, long-term data on sonidegib vs placebo trial

13.

Efficacy and safety of sonidegib in adult patients with nevoid basal cell carcinoma syndrome (Gorlin syndrome): Results from a phase 2, double-blind, randomized trial.

Lear J.T., Hauschild A., Stockfleth E., Squitieri N., Basset-Seguín N., Dummer R.

Clinical, Cosmetic and Investigational Dermatology. 13 (pp 117-121), 2020. Date of Publication: 2020.

➔ Included after eligibility assessment, short-term data on sonidegib vs placebo trial

44.

Canonical and non-canonical hedgehog signaling pathways: Role of G proteins.

Riobo N.A.

Topics in Medicinal Chemistry. 16 (pp 13-42), 2015. Date of Publication: 2015.

➔ Excluded after screening, review

52.

From an orphan disease to a generalized molecular mechanism: PTPN11 loss-of-function mutations in the pathogenesis of metachondromatosis.

Yang W., Neel B.G.

Rare Diseases. 1(OCT) (no pagination), 2013. Article Number: e26657. Date of Publication: 02 Oct 2013.

➔ Excluded after screening, different syndrome



## EMBASE ITRACONAZOLE AND BASAL CELL CARCINOMA

Database: Embase <1974 to 2021 Week 37>

Search Strategy:

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1 basal cell carcinoma/ (28620)

2 itraconazole/ (31765)

3 1 and 2 (165)

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Duplicates: 85

Excluded after screening: 79

Assessed: 1

Included: 1

31.

Topical Itraconazole for the Treatment of Basal Cell Carcinoma in Patients with Basal Cell Nevus Syndrome or High-Frequency Basal Cell Carcinomas: A Phase 2, Open-Label, Placebo-Controlled Trial.


Sohn G.K., Kwon G.P., Bailey-Healy I., Mirza A., Sarin K., Oro A., Tang J.Y.


JAMA Dermatology. 155(9) (pp 1078-1080), 2019. Date of Publication: September 2019.


➔ Included after eligibility assessment, results topical itraconazole in BCNS/mult BCC

**Supplementary Table 1.** Risk of bias of included efficacy trials

|   | Selection bias | Blinding of participants and personnel | Blinding of outcome assessment | Incomplete outcome data | Selective reporting | Other bias |
|---|----------------|--|--------------------------------|-------------------------|---------------------|------------|
| (Tang, Mackay-Wiggan et al. 2012, Tang, Ally et al. 2016) | +              | +                                      | +                              | ?                       | ?                   | +          |
| (Lear, Hauschild et al. 2020)                             | ?              | +                                      | +                              | ?                       | -                   | ?          |
| (Dreno, Kunstfeld et al. 2017)                            | +              | ?                                      | ?                              | +                       | +                   | +          |
| (Verkouteren, Wakkee et al. 2021)                         | +              | Not applicable                         | -                              | +                       | +                   | ?          |
| (Sohn, Kwon et al. 2019)                                  | +              | ?                                      | ?                              | +                       | +                   | +          |
| (Epstein, Lear et al. 2018)                               | +              | +                                      | +                              | ?                       | ?                   | ?          |
| (Skvara, Kalthoff et al. 2011)                            | +              | +                                      | +                              | ?                       | ?                   | +          |

 low risk of bias

 unknown risk of bias

 high risk of bias

## References

Dreno, B., et al. (2017). "Two intermittent vismodegib dosing regimens in patients with multiple basal-cell carcinomas (MIKIE): a randomised, regimen-controlled, double-blind, phase 2 trial." Lancet Oncol **18**(3): 404-412.

**BACKGROUND:** Vismodegib, a first-in-class Hedgehog-pathway inhibitor, is approved for use in adults with advanced basal-cell carcinoma. Patients with multiple basal-cell carcinomas, including those with basal-cell nevus (Gorlin) syndrome, need extended treatment. We assessed the safety and activity of two long-term intermittent vismodegib dosing regimens in patients with multiple basal-cell carcinomas. **METHODS:** In this randomised, regimen-controlled, double-blind, phase 2 trial, we enrolled adult patients with multiple basal-cell carcinomas, including those with basal-cell nevus syndrome, who had one or more histopathologically confirmed and at least six clinically evident basal-cell carcinomas. From a centralised randomisation schedule accessed via an interactive voice or web-based response system, patients were randomly assigned (1:1) to treatment group A (150 mg oral vismodegib per day for 12 weeks, then three rounds of 8 weeks of placebo daily followed by 12 weeks of 150 mg vismodegib daily) or treatment group B (150 mg oral vismodegib per day for 24 weeks, then three rounds of 8 weeks of placebo daily followed by 8 weeks of 150 mg vismodegib daily). Treatment assignment was stratified by diagnosis of basal-cell nevus syndrome, geographical region, and immunosuppression status. The primary endpoint was percentage reduction from baseline in the number of clinically evident basal-cell carcinomas at week 73. The primary analysis was by intention to treat. The safety population included all patients who received at least one dose of study drug. This trial is registered with ClinicalTrials.gov, number NCT01815840, and the study is ongoing. **FINDINGS:** Between April 30, 2013, and April 9, 2014, 229 patients were randomly assigned treatment, 116 in treatment group A and 113 in treatment group B. The mean number of basal-cell carcinoma lesions at week 73 was reduced from baseline by 62.7% (95% CI 53.0-72.3) in treatment group A and 54.0% (43.6-64.4) in treatment group B. 216 (95%) of 227 patients included in the safety analysis had at least one treatment-emergent adverse event deemed to be related to study treatment (107 [94%] of 114 in treatment group A and 109 [97%] of 113 in treatment group B). The most common grade 3 or worse treatment-related adverse events were muscle spasms (four [4%] patients in treatment group A vs 12 [11%] in treatment group B), increased blood creatine phosphokinase (one [1%] vs four [4%]), and hypophosphataemia (zero vs three [3%]). Serious treatment-emergent events were noted in 22 (19%) patients in treatment group A and 19 (17%) patients in treatment group B. Four (2%) patients died from adverse events; one (pulmonary embolism in treatment group A) was possibly related to treatment. **INTERPRETATION:** Both intermittent dosing schedules of vismodegib seemed to show good activity in long-term regimens in patients with multiple basal-cell carcinomas. Further study is warranted. **FUNDING:** F Hoffmann-La Roche.

Epstein, E. H., et al. (2018). "Hedgehog pathway inhibition by topical patidegib to reduce BCC burden in patients with basal cell nevus (Gorlin) syndrome." J Clin Oncol **36**(15).

Lear, J. T., et al. (2020). "Efficacy and Safety of Sonidegib in Adult Patients with Nevoid Basal Cell Carcinoma Syndrome (Gorlin Syndrome): Results from a Phase 2, Double-Blind, Randomized Trial." Clin Cosmet Investig Dermatol **13**: 117-121.

Skvara, H., et al. (2011). "Topical treatment of basal cell carcinomas in nevoid basal cell carcinoma syndrome with a smoothed inhibitor." J Invest Dermatol **131**: 1735-1744.

Sohn, G. K., et al. (2019). "Topical Itraconazole for the Treatment of Basal Cell Carcinoma in Patients With Basal Cell Nevus Syndrome or High-Frequency Basal Cell Carcinomas: A Phase 2, Open-Label, Placebo-Controlled Trial." JAMA Dermatol.

Tang, J. Y., et al. (2016). "Inhibition of the hedgehog pathway in patients with basal-cell nevus syndrome: final results from the multicentre, randomised, double-blind, placebo-controlled, phase 2 trial." Lancet Oncol **17**(12): 1720-1731.

**BACKGROUND:** Aberrant hedgehog signalling underlies the development of basal-cell carcinomas. We previously reported the interim analysis of a multicentre, randomised, double-blind, placebo-controlled, phase 2 trial in patients with the basal-cell nevus (Gorlin) syndrome indicating that the smoothed inhibitor vismodegib reduces basal-cell carcinoma tumour burden and prevents new basal-cell carcinoma growth in patients with basal-cell nevus syndrome. We report the final results of this 36 month trial. **METHODS:** In our multicentre, randomised, double-blind, placebo-controlled, phase 2 trial we enrolled patients aged 35-75 years with basal-cell nevus syndrome with at least ten surgically eligible basal-cell carcinomas at the Children's Hospital Oakland, Columbia University outpatient dermatology clinic (NY, USA) and a private practice outpatient dermatology office in Newport Beach (CA, USA). Patients were assigned to vismodegib or placebo (2:1) according to a randomisation sequence generated by computer code. The primary endpoint of the trial of 41 patients was to compare the effect of oral vismodegib (150 mg/day) versus placebo on the incidence of new surgically eligible basal-cell carcinomas after 3 months of treatment. In the subsequent, open-label phase (n=37) patients continued vismodegib at two sites for as long as month 36 (n=25) and at the third site were monitored up to month 36 (n=12). Additional endpoints for this phase were: whether continuous versus interrupted dosing differentially affected tumour burden; time to reach various levels of reduction in tumour burden; reduction in tumour size in patients who took less than 50% of the expected number of vismodegib tablets; reduction in the number of surgical excisions required per year before, during, and after treatment; and the effect of vismodegib on hedgehog target gene expression. We monitored patients at visits every 3 months for up to 36 months. The primary endpoint was analysed on a modified intention-to-treat basis. This trial is registered with ClinicalTrials.gov, number NCT00957229. **FINDINGS:** Between Sept 22, 2009, and Jan 24, 2011, 41 patients were monitored for a median of 36 months (IQR 36-36). Patients treated with vismodegib (n=26) had a mean reduced rate of new surgically eligible basal-cell carcinomas compared with patients randomly assigned to placebo (n=15; two [SD 0.12] new surgically eligible basal-cell carcinomas per patient per year vs 34 [1.32] new surgically eligible basal-cell carcinomas per patient per year, p<0.0001). In the 11 patients initially assigned to placebo, mean cross over to vismodegib reduced the development of new surgically eligible basal-cell carcinomas compared with placebo (0.4 [SD 0.2] new surgically eligible basal-cell carcinomas per patient per year vs 30.0 [7.8] new surgically eligible basal-cell carcinomas per patient per year, p<0.0001). Only three (17%) of 18 patients tolerated vismodegib continuously for the full 36 months. Fewer new surgically eligible basal-cell carcinomas developed in patients receiving vismodegib continuously than in those who interrupted dosing (mean 0.6 [0.72] new surgically eligible basal-cell carcinomas per patient per year vs 1.7 [1.8] new surgically eligible basal-cell carcinomas per patient per year, p<0.0001). Treatment-related grade 3-4 adverse events included weight loss of 20% or more (n=6) and muscle cramps (n=2). Two patients died during the course of the trial, one each from laryngeal and metastatic prostate cancer, deemed probably unrelated to drug. **INTERPRETATION:** Vismodegib reduces basal-cell carcinoma tumour burden in patients with basal-cell nevus syndrome. Adverse events associated with vismodegib frequently led to interruption of treatment, which is followed by basal-cell carcinoma recurrence. **FUNDING:** Genentech investigator-initiated trial funding, Clinical and Translational Science Award from the National Institutes of Health, National Institute of Arthritis and Musculoskeletal and Skin Diseases, National Cancer Institute, Damon Runyon Cancer Research Foundation Clinical

Investigator Award, Swim across America Foundation, and Michael J Rainen Family Foundation.

Tang, J. Y., et al. (2012). "Inhibiting the hedgehog pathway in patients with the basal-cell nevus syndrome." N Engl J Med **366**(23): 2180-2188.

**BACKGROUND:** Dysregulated hedgehog signaling is the pivotal molecular abnormality underlying basal-cell carcinomas. Vismodegib is a new orally administered hedgehog-pathway inhibitor that produces objective responses in locally advanced and metastatic basal-cell carcinomas. **METHODS:** We tested the anti-basal-cell carcinoma efficacy of vismodegib in a randomized, double-blind, placebo-controlled trial in patients with the basal-cell nevus syndrome at three clinical centers from September 2009 through January 2011. The primary end point was reduction in the incidence of new basal-cell carcinomas that were eligible for surgical resection (surgically eligible) with vismodegib versus placebo after 3 months; secondary end points included reduction in the size of existing basal-cell carcinomas. **RESULTS:** In 41 patients followed for a mean of 8 months (range, 1 to 15) after enrollment, the per-patient rate of new surgically eligible basal-cell carcinomas was lower with vismodegib than with placebo (2 vs. 29 cases per group per year,  $P < 0.001$ ), as was the size (percent change from baseline in the sum of the longest diameter) of existing clinically significant basal-cell carcinomas (-65% vs. -11%,  $P = 0.003$ ). In some patients, all basal-cell carcinomas clinically regressed. No tumors progressed during treatment with vismodegib. Patients receiving vismodegib routinely had grade 1 or 2 adverse events of loss of taste, muscle cramps, hair loss, and weight loss. Overall, 54% of patients (14 of 26) receiving vismodegib discontinued drug treatment owing to adverse events. At 1 month, vismodegib use had reduced the hedgehog target-gene expression by basal-cell carcinoma by 90% ( $P < 0.001$ ) and diminished tumor-cell proliferation, but apoptosis was not affected. No residual basal-cell carcinoma was detectable in 83% of biopsy samples taken from sites of clinically regressed basal-cell carcinomas. **CONCLUSIONS:** Vismodegib reduces the basal-cell carcinoma tumor burden and blocks growth of new basal-cell carcinomas in patients with the basal-cell nevus syndrome. The adverse events associated with treatment led to discontinuation in over half of treated patients. (Funded by Genentech and others; ClinicalTrials.gov number, NCT00957229.)

Verkouteren, B. J. A., et al. (2021). "Eight years of experience with vismodegib for advanced and multiple basal cell carcinoma patients in the Netherlands: a retrospective cohort study." Br J Cancer **124**(7): 1199-1206.

**BACKGROUND:** Vismodegib has been used for the treatment of locally advanced basal cell carcinoma (laBCC) and metastatic BCC (mBCC) since 2011. Most efficacy and safety data are provided by clinical trials. This study evaluates the effectiveness of vismodegib for the treatment of laBCC, mBCC and basal cell nevus syndrome (BCNS) patients, and the tumour characteristics associated with a higher probability of achieving a complete response in the Netherlands. **METHODS:** A retrospective cohort study that included all patients  $\geq 18$  years with histologically proven basal cell carcinoma that received  $\geq 1$  dose of vismodegib between July 2011 and September 2019 in the Netherlands. **RESULTS:** In total, 48 laBCC, 11 mBCC and 19 BCNS patients were included. Median progression-free survival was 10.3 months (95% confidence interval (CI), 7.5-22.6) for laBCC, 11.7 (95% CI, 5.2-17.5) for mBCC and 19.1 (95% CI, 7.4-20.2) for BCNS. Larger laBCCs were associated with a lower probability of complete response (hazard ratio (HR) 0.77 per increase in cm,  $p = 0.02$ ). Of all BCNS patients, 63% received  $\geq 2$  treatment sequences with vismodegib; all achieved partial responses. **CONCLUSIONS:** Half of the aBCC patients progress within 1 year after the start of vismodegib treatment. More research is needed to investigate other treatment strategies after vismodegib progression and to evaluate long-term effects of repetitive vismodegib treatment.



**Supplementary Table 2.** Oxford Center for Evidence-Based Medicine Levels

| Level | Therapy / Prevention, Aetiology / Harm                                    | Prognosis  | Diagnosis   | Differential diagnosis / symptom prevalence study    | Economic and decision analyses  |
|-------|---|--|---|--|---|
| 1a    | SR (with homogeneity*) of RCTs  | SR (with homogeneity*) of inception cohort studies; CDR" validated in different populations  | SR (with homogeneity*) of Level 1 diagnostic studies; CDR" with 1b studies from different clinical centres                              | SR (with homogeneity*) of prospective cohort studies | SR (with homogeneity*) of Level 1 economic studies  |
| 1b    | Individual RCT (with narrow Confidence Interval");                        | Individual inception cohort study with > 80% follow-up; CDR" validated in a single population  | Validating** cohort study with good" " " reference standards; or CDR" tested within one clinical centre                                 | Prospective cohort study with good follow-up****     | Analysis based on clinically sensible costs or alternatives; systematic review(s) of the evidence; and including multi-way sensitivity analyses                 |
| 1c    | All or none§  | All or none case-series  | Absolute SpPins and SnNouts" "  | All or none case-series                              | Absolute better-value or worse-value analyses " " " "   |
| 2a    | SR (with homogeneity*) of cohort studies                                  | SR (with homogeneity*) of either retrospective cohort studies or untreated control groups in RCTs  | SR (with homogeneity*) of Level >2 diagnostic studies   | SR (with homogeneity*) of 2b and better studies      | SR (with homogeneity*) of Level >2 economic studies   |
| 2b    | Individual cohort study (including low quality RCT; e.g., <80% follow-up) | Retrospective cohort study or follow-up of untreated control patients in an RCT; Derivation of CDR" or validated on split-sample§§§ only | Exploratory** cohort study with good" " " reference standards; CDR" after derivation, or validated only on split-sample§§§ or databases | Retrospective cohort study, or poor follow-up        | Analysis based on clinically sensible costs or alternatives; limited review(s) of the evidence, or single studies; and including multi-way sensitivity analyses |

|    |   |   |   |   |  |
|----|---|---|---|---|--|
| 2c | “Outcomes” Research;<br>Ecological studies  | “Outcomes” Research   |   | Ecological studies  | Audit or outcomes research   |
| 3a | SR (with homogeneity*)<br>of case-control studies   |   | SR (with homogeneity*) of 3b<br>and better studies  | SR (with homogeneity*)<br>of 3b and better studies  | SR (with homogeneity*) of<br>3b and better studies   |
| 3b | Individual Case-<br>Control Study   |   | Non-consecutive study; or<br>without consistently applied<br>reference standards  | Non-consecutive<br>cohort study, or very<br>limited population  | Analysis based on limited<br>alternatives or costs, poor<br>quality estimates of data,<br>but including sensitivity<br>analyses incorporating<br>clinically sensible variations. |
| 4  | Case-series (and poor<br>quality cohort and case-<br>control studies§§)   | Case-series (and poor<br>quality prognostic cohort<br>studies***)   | Case-control study, poor or<br>non-independent reference<br>standard  | Case-series or<br>superseded reference<br>standards   | Analysis with no sensitivity<br>analysis   |
| 5  | Expert opinion without<br>explicit critical<br>appraisal, or based on<br>physiology, bench<br>research or “first<br>principles” | Expert opinion without<br>explicit critical appraisal, or<br>based on physiology, bench<br>research or “first principles” | Expert opinion without explicit<br>critical appraisal, or based on<br>physiology, bench research or<br>“first principles” | Expert opinion without<br>explicit critical<br>appraisal, or based on<br>physiology, bench<br>research or “first<br>principles” | Expert opinion without<br>explicit critical appraisal, or<br>based on economic theory or<br>“first principles”   |



