

# CASE REPORT: USE OF VISMODEGIB IN A PATIENT WITH GORLIN GOLTZ SYNDROME

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## Background:

Gorlin Goltz Syndrome (SGG) is caused by the mutation of the Hedgehog (Hh) gene. The clinical consequences are related to the production of basal cell carcinomas (BCC) and alteration of normal tissue development. Vismodegib is a drug that inhibits the Hh signaling pathway by blocking the activation of the transmembrane-7 Smoot-hened receptor.

## Purpose:

Evaluation of the effectiveness and safety of vismodegib in a patient with BCC caused by SGG

### Material and methods:

Follow-up of a patient treated with vismodegib a cause of multiple CBCs. Previous treatment lines, the size and number of BCCs, the suitability of vismodegib in patients with SGG and the adverse effects presented were reviewed.

## Results:

A 62-year-old patient was diagnosed with SGG at 22 years of age. The patient's multiple CBCs from the youth were controlled by surgical interventions, topical 5-fluorouracil, imiquimod, retinoids and photodynamic therapy.

In 2016, before the impossibility of submitting the patient with new excision, treatment with vismodegib 150 mg starting every 24h. The effectiveness of the treatment has been crucial due to the fact that the second month had a reduction of BBC > 30%.

During the 13 months of treatment with vismodegib, the patient presented adverse reactions such as joint pain and generalized tiredness throughout the treatment and a punctual increase in the values of the liver enzymes that conditioned the suspension for 10 days.

After 10 months of the starting treatment, as a consequence of the adverse effects and at the request of the patient, a therapeutic rest of 2 months prescribed, restarting again due to the risk of relapse.

## Conclusion:

Although we are faced with a syndrome of low incidence and with few studies in terms of available treatments, we have made possible a significant decrease in BCC. Regarding the duration of treatment, the ERIVANCE fundamental study presented a median treatment duration of 9.6 months, having been an overcome in our case.

All adverse effects presented in the technical file, highlighting the fatigue and joint pain that have conditioned the therapeutic rest

## References:

Sekulic A, Migden MR, Lewis K, et al. Pivotal ERIVANCE basalcell carcinoma (BCC) study:12-month update of efficacy and safety of vismodegib in advanced BCC. J Am Acad Dermatol.2015;72(6):1021-1026.e8

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