ASSESSING PAYER PERSPECTIVES ON NOVEL THERAPIES FOR THE TREATMENT OF GORLIN SYNDROME

HSD94

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INTRODUCTION

Gorlin Syndrome (GS) is a rare genetic cancer syndrome characterized by multiple early onset basal cell carcinomas (BCC), odontogenic keratocysts and other abnormalities[1].

Management of BCCs in GS patients typically involves a mixture of topical and/or systemic treatments along with methods to physically remove the lesions[1-3].

OBJECTIVES

The aim was to explore payer knowledge, perception, and unmet needs related to GS and assess their expectations and evaluation criteria for novel topical therapies.

METHODS

Comprehensive landscape analysis of the current GS treatment

Including the evaluation of existing therapies, their efficacy, and the associated economic burden

Primary research: payers' interviews

A qualitative analysis was conducted from semi-structured interviews with 14 payers, focused on understanding their perspectives on novel therapies for GS.

- 3 Germany
- 3 UK
- 3 Spain
- 5 USA

Secondary research

Secondary resources such as payer decisions, case studies, and professional journals were also reviewed.

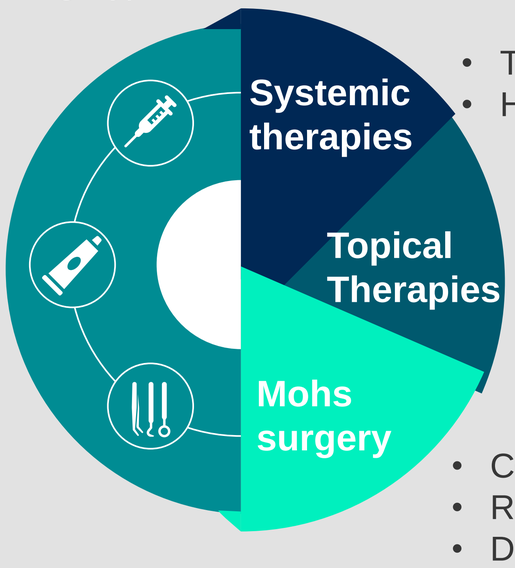
RESULTS

Clinical burden

Payers recognized the high clinical burden and negative impact on patient quality of life (QoL) associated with GS and its repeated need to remove the lesions, the pain and the risk of infection, cancer or disfigurement.

Unmet needs

When evaluating the current treatment options (hedgehog inhibitors, imiquimod, fluorouracilo, photodynamic therapy, mohs surgery) some unmet needs were identified by the payers:



- Tolerability could be improved
- High cost

- Limited efficacy
- Cumbersome to use

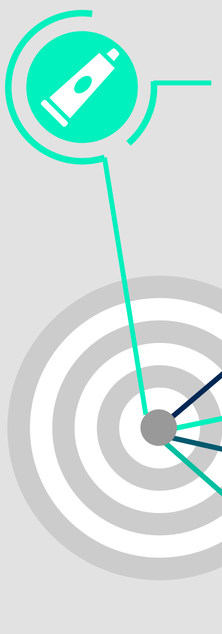
- Cornerstone of treatment
- Risk of disfigurement
- Difficulties in sensitive lesions close to the eyes, lips or nose
- Economic implications

Payers expressed a **need for a treatment** that could reduce the **number and frequency of surgeries**, while **improving patient QoL and convenience**.

Clinical Unmet Needs	Economic Unmet Needs
Reduce the number and frequency of surgical procedures	Reduce the number and frequency of surgical procedures
Avoid procedures that could lead to disfiguration	Reduce the need for expensive reconstructive surgery
Reduce potential transformation to locally advanced or malignant lesions	Reduce the need for expensive Hedgehog inhibitors or chemotherapies
Reduce the recurrence of lesions	Reduce the number of visits to health professionals / HRU

Improve de convenience, tolerability → Improvement in QoL

Potential for novel topical therapies



According to payers, they saw the **potential for novel topical therapies** to target sensitive lesions difficult to remove surgically with neo-adjuvant or stabilizing topical therapies, thereby reducing the risk of progression to malignant or more complex lesions.

Differential dimensions of differentiation in GS according to payers

		Level of agreement among payers
Location of lesions / risk of disfigurement	The main differentiation dimension consistently mentioned by payers. Perceived to impact the therapeutic approach and potentially the cost of therapy if there is need for reconstructive surgery.	
Distribution of lesions	Also mentioned by most interviewees. Lesions on the face and other visible parts of the body are considered to have a higher impact on QoL for potential cosmetic outcomes after surgery.	
Risk of lesion transformation	Lesions transforming into malignant have higher clinical burden and are more expensive to treat. However, payers are unclear on how to rate the risk of transformation in GS.	
Others	GS is more impactful on children due to both impact on QoL and cumulative expected lifetime number of lesions.	

CONCLUSIONS

- GS poses significant challenges in terms of clinical burden, impact on QoL and economic implications.
- From a payer perspective, there is a clear unmet need to explore alternative treatment approaches that can reduce the number and frequency of surgeries to protect the physical integrity of patients, improve the tolerability and convenience while improving their QoL.
- All these factors present an opportunity for new topical therapies that can effectively address this unmet need, bridging the gap in treatment options for GS.

REFERENCES: 1. Lo Muzio L,.Nevoid basal cell carcinoma syndrome (Gorlin syndrome). Orphanet J Rare Dis 2008;3:32.; 2. Evans DG, Farndon PA. Nevoid Basal Cell Carcinoma Syndrome. In: Adam MP, Ardinger HH, Pagon RA, et al., editors. GeneReviews. 2002 Jun 20; 3. Gorlin, R. Nevoid basal cell carcinoma (Gorlin) syndrome. Genet Med 6, 530–539 (2004).

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