Optum Frontier Therapies™

Moving towards meaningful patient outcomes in rare disease: Achondroplasia case study

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Introduction

The primary outcome measurement in clinical trials for achondroplasia treatment, vosoritide, was growth velocity. The Federal Drug Administration (FDA) approved vosoritide based on data provided. Data informed stakeholders about the effectiveness of the medication, but not necessarily what the community of people impacted by achondroplasia nor all prescribers considered the primary concern: the correlation between treatment and fewer medical interventions for life-threatening complications of achondroplasia.

Our objectives of this case study were three-fold:

1. Review the gap between clinical trials data for drug approval and the real-world outcomes that patients, caregivers and prescribers' want.
2. Design a novel approach to making decisions about patient care.
3. Achieve meaningful patient outcomes for first-in-class therapy for achondroplasia.

Methods

Using a simple randomization method, 10% of the prescribers from the pivotal phase 3 clinical trial were selected to be interviewed.[1] We also screened patient advocacy groups by using the National Center for Advancing Translational Sciences approved advocacy groups and Little People of America.

We asked key stakeholders: What is the most important outcome to collect for patients considering treatment for achondroplasia? Our team conducted interviews with:

- Key opinion leaders including a geneticist, pediatricians and an orthopedic surgeon
- Patient support group/biotech council, the Little People of America Biotech Committee

Results

Our team captured the following outcomes which, except for the depression scales, were important to all stakeholders:

- Prevalence of sleep apnea
- Number of ear infections
- Spinal stenosis and foramen magnum surgeries related to achondroplasia
- Pain scale
- Patient health questionnaire (PHQ)2 and PHQ9 depression scales*
- Growth velocity

Conclusion

By partnering and collaborating with the real experts – patients, caregivers, key opinion leaders and prescribers – Optum® Frontier Therapies created a patient-first outcomes model that will help people make decisions regarding treatment. We continue to conduct extensive research with patient advocacy groups, payers, prescribers and manufacturers to continue to inform our approach and include the voice of the patient.[2] We look forward to presenting our data within the next 12 months.

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Reference: 1. Savarirayan R, Tofts L, Irving M, et al. Once-daily, subcutaneous vosoritide therapy in children with achondroplasia: a randomised, double-blind, phase 3, placebo-controlled, multicentre trial. Lancet. 2020; 396(10252):684-692. Published correction appears in Lancet. 2020 Oct 10; 396(10257):1070. | 2. Kayyali R, Gebara SN, Hesso I, et al. Shared decision making and experiences of patients with long-term conditions: has anything changed? BMC Health Ser Res. 2018; 18(1):763.

*Little People of America advocated for inclusion of the depression scales