Clinical Program in Achondroplasia: Overview

QED Therapeutics, a BridgeBio Company, is developing infigratinib, an orally bioavailable investigational drug being studied for the treatment of children with achondroplasia. Infigratinib has not been approved for use or determined to be safe or effective in human children with achondroplasia.

PROPEL Study Overview

The PROPEL study is an ongoing prospective, non-interventional study of children with achondroplasia.

This study is collecting information on the health and growth of children with achondroplasia. No investigational treatment is given as part of this study. Children who complete the PROPEL study may have the option to enroll in a QED Therapeutics-sponsored interventional study.

Study Eligibility

The PROPEL study is enrolling children between the ages of 2.5 and 10 years old (inclusive) who have been diagnosed with achondroplasia. Participants must:

- Have a confirmed diagnosis of achondroplasia
- Be able to stand without assistance
- Not have hypochondroplasia or short stature condition other than achondroplasia
- Not have been treated with growth hormone or any medication in development for achondroplasia

For a complete list of inclusion criteria for the PROPEL study, visit ClinicalTrials.gov and search for code NCT04035811.

The *primary endpoint* measured in this observational study is the rate of growth (annualized height velocity). Additional outcome measures seek to better understand the overall health and medical complications that occur in children with achondroplasia.

Participation in PROPEL is planned for six months to two years. Children participating in the the study will visit the study site at month 3,month 6 and every six months thereafter until study completion.

PROPEL 2 Trial Overview

PROPEL 2 is a prospective, Phase 2, open-label global interventional trial to obtain the preliminary evidence of safety and efficacy of infigratinib in children with achondroplasia and to determine the dose of infigratinib for future studies. Eligible children would have had to complete at least six months in PROPEL before participating in this study.

The *primary endpoints* are safety and change from baseline in annualized height velocity.

Additional endpoints evaluated include:

- Changes in body proportions
- Quality of life
- Changes in occurrence of medical events observed in children with achondroplasia

Children in this study will receive infigratinib orally once daily for up to 18 months. All children who complete the PROPEL 2 Trial will have the opportunity to continue receiving infigratinib in a long-term extension trial.

For more detailed information on PROPEL 2, visit ClinicalTrials.gov and search for code NCT04265651.

Note: The safety and efficacy of infigratinib have not been demonstrated for the use described here. There is no guarantee that infigratinib will receive health authority approval or become commercially available in any county for the uses being investigated.

Participating Sites and Locations

United States

California
- Benioff Children's Hospital Oakland; Oakland, CA 94609

Delaware
- Nemours Alfred I. Dupont Hospital for Children; Wilmington, DE 19803

Ohio
- Cincinnati Children's Hospital; Cincinnati, OH 45229

Tennessee
- Vanderbilt University Medical Center; Nashville, TN 37232

Texas
- Cook's Children Medical Center; Dallas, TX 75207

Global

Australia
- Murdoch Children's Research Institute; Parkville, Australia

Canada, Alberta
- Stollery Children's Hospital; Edmonton, Alberta, Canada T6G 2H7

France
- Hospital Femme Mere Enfant; Lyon, France
- Hospital Necker-Enfants Malades; Paris, France
- Hospital des Enfants; Toulouse, France

Spain
- Vithas Hospital San Jose; Barcelona, Spain 08305
- Hospital Universitario La Paz; Madrid, Spain 24086
- Hospital Universitario Virgen de la Victoria, Malaga, Spain

United Kingdom
- Bristol Royal Hospital for Children; Bristol, England, UK
- Birmingham Children's Hospital; Birmingham, UK
- Queen Elizabeth University Hospital; Glasgow, UK
- St. Thomas' Hospital; London, UK
- Manchester University Children's Hospital; Manchester, UK
- Sheffield Children's Hospital; Sheffield, UK