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Medical History of Children Enrolled in PROPEL: A Prospective Clinical Assessment Study in Children with Achondroplasia

Melita Irving, MBBS; MD; FRCPCH, Josep Maria De Bergua, MD, Daniela Rogoff, MD, PhD, Ravi Savarirayan, MB, BS, MD, FRACP, ARCPA, Paul Arundel, MBBS DCH, Jean Pierre Salles, MD, PhD, Antonio Leiva-Gea, MD, Vrinda Saraff, MBBS MRCPCH MSc, Helen McDevitt, MbCHB MD, Fernando Santos-Simarro, MD, Marc Nicolino, MD, PhD, Valerie Cormier-Daire, MD, PhD, Peter Kannu, MB, ChB, PhD, FRACP, FRCPC, Mars Skae, MbCHB, MPhil, Michael B. Bober, MD, PhD, John Phillips III, MD, Christine Burren, MBBS, MD, FRACP, FRCPCH, Paul Harmatz, MD, Howard Saal, MD, Julie Hoover-Fong, MD, PhD, FACMGG, Richard Weng, MS, Elena Muslimova, MD, PhD, and Terry Cho, BA

Background: Achondroplasia (ACH) is the most common short-limbed skeletal dysplasia, affecting between 1 in 15,000 to 1 in 30,000 live births. People with ACH are at risk for several significant co-morbidities, including foramen magnum stenosis, obstructive sleep apnea, chronic otitis media with conductive hearing loss, spinal stenosis, and a propensity towards obesity. PROPEL is a prospective, non-interventional study designed to examine baseline growth parameters and health status in children being assessed for potential enrollment into interventional studies with infigratinib, an oral FGFR1–3 inhibitor in development as a therapeutic option for ACH. Here we describe the medical complications reported as medical history in the PROPEL study.

Methods: Children with ACH between the ages of 2.5 and 10 years are eligible for enrollment in PROPEL and are evaluated at screening/baseline, month 3, month 6, and every 6 months thereafter. Medical history collected at screening/baseline is summarized using system organ class and preferred terms.

Results: A total of 86 children with ACH (60% female, mean±SD age 6.1±2.5 years) have been enrolled to date at 19 sites in Europe, Australia and North America. Fifty-eight children had undergone surgical and medical procedures with a mean of 2.9 procedures per child (1–11 surgeries/subject). The most common procedures were pressure-equalizing ear tube insertion, adenoidectomy and tonsillectomy. Twenty-one (24%) children had undergone at least 1 surgery (1–5 surgeries/child) for spine or cranial decompression. History of infections and respiratory disorders were reported in 46 (53%) and 40 (47%) children, respectively, the most common being ear infections and obstructive sleep apnea. Musculoskeletal disorders were described in 33 (38%) children, with kyphosis being the most common. Hydrocephalus was reported in 2 children, while 4 had ventriculomegaly without intracranial hypertension. Congenital cardiovascular abnormalities were found in 4 children, 2 of whom presented with patent ductus arteriosus and 2 had patent foramen ovale. A comprehensive summary of medical histories will be presented at the conference.

Conclusions: The PROPEL study has a planned total enrollment of 200 children and seeks to contribute to the deeper understanding of the natural history of ACH. Data described here highlight the significant complications and high number of interventions that children with ACH undergo throughout infancy and childhood. This stresses the importance of expert management of this complex condition.

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