InnoSkel awarded Innovation Passport by the UK MHRA for the treatment of a rare bone disorder

First step in the recent UK MHRA initiative to accelerate the development and access to innovative medicines in the UK

Designation recognizes the potential of InnoSkel's lead program for pediatric patients with spondyloepiphyseal dysplasia congenita (SEDc)

Nice, France, 16 December 2021 – InnoSkel (the "Company"), a pioneering biotechnology company developing transformative therapies for the unmet needs of individuals with rare bone disorders, today announced that it has been awarded an Innovation Passport under the UK Medicines and Healthcare products Regulatory Agency's (MHRA) Innovative Licensing and Access Pathway (ILAP), to pursue accelerated patient access for its first-in-class treatment for spondyloepiphyseal dysplasia congenita (SEDc), a type II collagen disorder.

INS-101, InnoSkel's lead gene therapy program, is being developed for the treatment of SEDc in pediatric patients, for whom there is currently no disease-modifying therapy available. SEDc is a rare genetic bone disorder that is present from birth and is associated with skeletal deformities that worsen with age, some of which can be life-threatening requiring repeat surgical procedures. Patients with SEDc also experience a number of other severe complications, including irregular bone growth, respiratory insufficiency, joint pain, hip and spine deformities and early onset of osteoarthritis. INS-101 is designed to be administered by a systemic delivery route to restore COL2A1 function in growth plates, making it an ideal candidate to treat SEDc. INS-101 has demonstrated strong efficacy in a mouse model, showing restoration of bone growth and prevention of disease complications.

Elvire Gouze, PhD, Founder and Chief Executive Officer of InnoSkel, said: *"The Innovation Passport designation by the UK MHRA is an important milestone for InnoSkel and the SEDc community and will enable us to accelerate the development of a treatment for this rare and debilitating bone disorder. The passport provides the opportunity to engage with regulators, health technology assessment (HTA) bodies and other partners at a pre-clinical stage with the endorsement of an acceptable risk assessment. In addition, the target development profile (TDP) will assist the company with the clinical trial design and evidence generation with the aim of achieving early access in the UK."*

The MHRA's ILAP was launched at the start of 2021 and aims to accelerate the time to market, facilitating safe, early and financially sustainable patient access to medicines. The pathway features enhanced input and interactions with the MHRA and other stakeholders including the National Institute for Health and Care Excellence (NICE), and the Scottish Medicines Consortium (SMC). The decision to award the Innovation Passport to InnoSkel's INS-101 program was made by the ILAP Steering Group, which is comprised of representatives from MHRA, NICE, and SMC.

To receive an Innovation Passport, INS-101 met the following criteria: (1) the condition is life-threatening or seriously debilitating; (2) the program is intended for a rare disease or special population; and (3) the medicine has the potential to offer benefits to patients.

The Innovation Passport designation is the first step in the ILAP process and triggers the MHRA and its partner agencies to create a target development profile to chart out a roadmap for regulatory and development milestones with the goal of early patient access in the UK. Other benefits of ILAP include a 150-day accelerated assessment, rolling review and a continuous benefit risk assessment.

Ends

About SEDc

SEDc is a rare disease with a prevalence of 1 in 50,000. It is the most common severe type II collagen disorder, resulting from mutations in the COL2A1 gene, which prevents bones and other connective tissues from developing properly. Clinical features of type II collagen disorders show a wide range of severity and complexity. SEDc typically presents with severe disproportionate short stature with high risk of cervical instability, spinal cord damage, hip joint deformity, locomotor difficulties, respiratory insufficiency, ocular manifestations, orofacial features and premature osteoarthritis.

About InnoSkel

InnoSkel is a pioneering platform biotechnology company developing transformative therapies for the unmet needs of individuals with rare bone disorders. Initially the Company is developing a gene therapy for a group of diseases known as type II collagen disorders, whilst also expanding the use of its technology and know-how to target and treat other rare bone conditions. InnoSkel's lead asset, INS-101, is a gene therapy for type II collagen disorders which has demonstrated good efficacy in proof-of-concept studies. The Company's goal is to make a meaningful difference to the lives of underserved patient populations suffering from debilitating bone disorders. InnoSkel's labs are in Sophia Antipolis Biotechnology Park in Nice, France.

For more information, visit www.innoskel.com

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