Press Release
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# FDA grants Medivir's MIV-711 Rare Pediatric Disease Designation for Osteogenesis Imperfecta

**Stockholm — Medivir AB (Nasdaq Stockholm: MVIR), a pharmaceutical company focused on developing innovative treatments in areas of high unmet medical need, announced today that the US Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation (RPDD) to its selective cathepsin K inhibitor MIV-711 for the treatment of Osteogenesis Imperfecta (OI). MIV-711 already holds Orphan Drug Designation (ODD) in OI, as well as ODD and RPDD in Perthes disease. The decision strengthens MIV-711 ahead of the planned proof-of-concept study in OI and, upon a future approval, MIV-711 may also qualify for a Priority Review Voucher – a transferable asset that has sold for USD 180–210 million per voucher in 2026.**

**MIV-711 targets the underlying bone fragility**
MIV-711 inhibits cathepsin K, the main enzyme used by osteoclasts to degrade collagen in bone tissue. By reducing excessive bone resorption, MIV-711 aims to restore the balance between bone resorption and bone formation, thereby preventing fractures and skeletal deformities.

For RPDD, the FDA requires a scientific rationale and supportive data suggesting that the drug may be effective in the disease. In an OI-specific animal model, MIV-711 has shown significant and dose-dependent improvements in bone strength, bone quality and bone morphology, and clinical data in osteoarthritis support MIV-711's ability to counteract bone degradation in humans. In its decision, the FDA concluded that OI meets the criteria for a rare pediatric disease, citing, among other things, high bone fragility with susceptibility to fracture, skeletal deformities, respiratory complications, pain and short stature.

There is currently no approved treatment that addresses the underlying bone fragility in Osteogenesis Imperfecta, and Medivir estimates the market potential for such a treatment at a minimum of USD 2.5 billion.

**Priority Review Voucher – a potentially valuable asset**
With RPDD, the FDA may grant Medivir a Priority Review Voucher (PRV) upon marketing approval of MIV-711 in OI. The voucher provides priority review of a subsequent marketing application and can be sold on to other companies. Transactions completed in 2026 have been concluded at between USD 180 million and USD 210 million per voucher. The PRV program has been extended several times since its introduction in 2012, most recently in February 2026, and currently runs until September 30, 2029.

- *"The FDA's decision to grant RPDD in Osteogenesis Imperfecta confirms both the significant unmet medical need and the scientific rationale for MIV-711. Children with OI live with recurrent fractures, skeletal deformities and chronic pain, and there is still no approved treatment that addresses the underlying bone fragility. We believe MIV-711 has the potential to become the first. With Orphan Drug Designation and RPDD in both OI and Perthes disease, a finalized study design and secured funding, MIV-711 is well positioned for the next phase of development. Our focus now is on initiating the proof-of-concept study and demonstrating what MIV-711 can mean for patients," says Jens Lindberg, CEO, Medivir.*

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**For additional information, please contact:**
Jens Lindberg, CEO, Medivir AB
Telephone: +46 8 5468 3100
E-mail: jens.lindberg@medivir.com

**About MIV-711**
MIV-711 is a potent and selective inhibitor of cathepsin K, the main protease involved in breaking down collagen in bone and cartilage. It has been shown to slow, stop or reverse the progressive degeneration of joints affected by osteoarthritis. By inhibiting cathepsin K and increased/excessive osteoclast activity, MIV-711 has the potential to counteract the excessive bone breakdown seen in patients with Osteogenesis Imperfecta (brittle bone disease). MIV-711 restores the balance between the breakdown of bone with mutated collagen and the formation of new bone with the aim of preventing fractures and bone deformities.

**About Osteogenesis Imperfecta**
Osteogenesis imperfecta (OI), also known as brittle bone disease, is a rare and hereditary disorder characterised by fragile bones with a high susceptibility to fractures. The disease is caused by various genetic mutations that affect the structure or production of the bone matrix. The severity varies from mild to very severe forms. The most severe cases can involve repeated fractures, skeletal deformities, pain and short stature, and in the most severe form, children do not survive infancy. Globally, an estimated 500,000 people are affected, of whom around 135,000 are children. OI often requires lifelong treatment, with the aim of improving quality of life and reducing the risk of fractures.

**About Medivir**
Medivir develops innovative therapies targeting diseases with high unmet medical need. The company's drug candidates focus on indications where current treatment options are limited or non-existent, offering the potential to deliver meaningful improvements for patients. Medivir's two lead programs are fostrox, a precision chemotherapy designed to selectively target liver cancer cells while minimizing side effects, and MIV-711, aimed at treating Osteogenesis Imperfecta (Brittle bone disease) and Legg-Calvé-Perthes disease (Perthes disease). Both candidates have blockbuster potential, representing significant value creation opportunities Medivir's shareholders. Collaborations play a key role in Medivir's business model, with drug development conducted either in-house or in partnership. Medivir (Nasdaq Stockholm: MVIR) is listed on the Small Cap segment of Nasdaq Stockholm. More information is available at www.medivir.com