Press Release
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MEDIVIR

FDA grants Medivir´s MIV-711 Orphan Drug Designation for the treatment of Osteogenesis Imperfecta

Stockholm, Sweden — Medivir AB (Nasdaq Stockholm: MVIR), a pharmaceutical company focused on developing innovative treatments for cancer in areas of high unmet medical need, announced today that its selective cathepsin K inhibitor, MIV-711, has been granted Orphan Drug Designation (ODD) by the FDA for the treatment of Osteogenesis Imperfecta (OI).

Orphan drug designation is granted to investigational treatments for rare diseases that affect fewer than 200,000 people in the United States. The designation provides assistance in developing drugs, tax credits, exemptions from FDA fees and 7 years of marketing exclusivity.

- “OI is a rare, genetic disorder that negatively affects the body’s ability to produce type I collagen which leads to bone fragility causing bone deformities and frequent fractures, often with minimal or no trauma. There are multiple subtypes of OI ranging from Mild to Severe where the most severe types are not compatible with life and patients die shortly after birth having suffered from multiple fractures. There is a significant unmet medical need as there are no approved treatment options for patients diagnosed with OI. We are delighted that MIV-711 has been granted ODD by the FDA, which strengthens the evidence base for its ability to positively impact bone remodeling and its potential to treat bone-related disorders”, said Jens Lindberg, CEO of Medivir.

To gain ODD, supportive data suggesting that the drug may be effective in the disease is required. MIV-711 has shown, in an OI-specific animal model, significant and dose-dependent positive impact on bone strength and quality as well as bone morphology, supporting potential benefit in treating patients with OI. Clinical data in osteoarthritis further support that MIV-711 has a positive effect in the prevention of bone degradation, translating to potential clinical benefit in OI.

MIV-711 inhibits cathepsin K, which is the main enzyme by which osteoclasts, that are involved in bone resorption and cartilage degradation, cleave collagen in the bone matrix. Cathepsin K inhibition protects the damaged bone by reducing bone resorption and promoting bone formation, thereby addressing the key mechanisms causing pathological changes in OI, with potential to minimize long-term negative effects.

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About Osteogenesis Imperfecta

Osteogenesis imperfecta (OI), commonly known as brittle bone disease, is a genetic disorder characterized by fragile bones that break easily, often with little or no trauma. OI is a group of genetic disorders that primarily affect the bones, leading to increased fragility and a higher risk of fractures. The condition is caused by genetic mutations resulting in defects in the structure or production of collagen, particularly type I collagen, which is essential for bone strength and structure. OI can vary significantly in severity, with symptoms ranging from mild to severe. It is a lifelong condition that requires ongoing management to improve quality of life and reduce the risk of fractures.

About MIV-711

MIV-711 is a potent and selective inhibitor of cathepsin K, the principal protease involved in breaking down collagen in bone and cartilage. MIV-711 has been shown to slow, stop or reverse the progressive degeneration of joints affected by osteoarthritis. By inhibiting cathepsin K and increased/excessive activity of osteoclasts, MIV-711 has the potential to counteract excessive bone resorption. MIV-711 restores the balance between excessive bone resorption and bone formation with the aim of preventing fractures and bone deformities.

About Medivir

Medivir develops innovative drugs with a focus on cancer where the unmet medical needs are high. The drug candidates are directed toward indication areas where available therapies are limited or missing and there are great opportunities to offer significant improvements to patients. Medivir is focusing on the development of fostroxacitabine bralpamide (fostrox), a drug candidate designed to selectively treat cancer cells in the liver and to minimize side effects. Collaborations and partnerships are important parts of Medivir's business model, and the drug development is conducted either by Medivir or in partnership. Medivir's share (ticker: MVIR) is listed on Nasdaq Stockholm's Small Cap list. www.medivir.com.