

Medivir announces positive topline results from phase IIa osteoarthritis study, showing disease-modifying benefit of MIV-711 on joint structure

Stockholm, Sweden — Medivir AB (Nasdaq Stockholm: MVIR) today announces positive top line data from MIV-711-201, the 6-month initial phase IIa study of MIV-711 in patients with moderate knee osteoarthritis. MIV-711, which is being developed as a disease-modifying osteoarthritis drug (DMOAD), demonstrated benefit on joint structure in the study. Patients who received MIV-711 for 6 months had significantly lower increases in bone area and cartilage thinning in the diseased knee compared to patients who received placebo. With clinical data demonstrating MIV-711's potential to be the first disease modifying drug for osteoarthritis, Medivir has retained strategic advisors to seek a partner for the future development of MIV-711.

INVITATION TO CONFERENCE CALL AND WEBCAST

Medivir will be presenting the top line data from the study on a conference call/webcast Tuesday, 26 September, at 14.00 CET. Details on how to participate are found below.

Increasing joint bone area and thinning of the joint cartilage in the femur, assessed using magnetic resonance imaging (MRI), are measures of the ongoing degenerative changes in the joint and have been shown to correlate well with overall clinical disease progression in osteoarthritis patients^{1,2}. The same imaging techniques, which are objective measures of disease progression, were used to assess changes in joint structure in the MIV-711-201 study:

- Patients receiving MIV-711 once daily at both 100 and 200 mg doses experienced approximately 65% reductions in femoral joint bone area progression in the 6-month period compared to those receiving placebo (unadjusted p-values for both doses < 0.005). Similar to previous epidemiological cohort studies, patients who received placebo in this study showed a 1% increase in medial femur joint bone area over the 6-month treatment period.
- MIV-711 also showed a benefit on cartilage degradation, with the 100mg group experiencing a 70% reduction in median loss of femoral cartilage thickness relative to placebo group, and the 200mg group even showing a small increase in median cartilage thickness.

MIV-711 did not show a statistically significant effect on patient-reported numerical rating scale (NRS) pain following 6 months of treatment, the primary endpoint of the study. Nevertheless, consistent tendencies favouring both the 100mg and 200mg groups were observed across patient-reported pain and other patient-reported symptoms.

The study data also indicate that both MIV-711 doses showed acceptable safety and tolerability for this patient population.

"The finding that MIV-711 slows the degenerative changes on both bone and cartilage in knees affected by osteoarthritis is an enormously exciting finding" said Professor Philip Conaghan, Professor of Musculoskeletal Medicine at the University of Leeds, UK, and lead investigator on the MIV-711-201 study. "Long-term studies in osteoarthritis patients have shown that the progression of degenerative changes in diseased joints measured by MRI is correlated with subsequent total joint replacement. Although MIV-711 didn't show a statistically significant improvement on pain in this limited-duration study, the effects on joint structure and trends in symptoms observed after 6 months should result in significant clinical benefit if sustained over a longer period of time. With no disease modifying drugs currently available, osteoarthritis patients are in

urgent need of medications that can slow or halt the progression of joint damage. The findings from this study, in which we showed structural benefit with an orally administered drug after only 6 months of treatment are unique and show promise for the future of osteoarthritis treatment.”

Detailed data from the study will be submitted for presentation at an upcoming scientific meeting.

“This unprecedented data showing the beneficial effects on joint structure degeneration, coupled with its acceptable safety profile strongly support the further development of MIV-711 as a disease-modifying osteoarthritis drug” said Christine Lind, Medivir CEO. “As Medivir remains focused on its portfolio of oncology projects, we will be initiating efforts to find a partner to advance the development of MIV-711 and bring this promising treatment to osteoarthritis patients.”

Medivir has initiated a process to seek a partner for future MIV-711 development and has retained Greenhill & Co. as advisors.

INVITATION TO CONFERENCE CALL AND WEBCAST

Conference call/webcast to be held Tuesday, 26 September, at 14.00 CET.

Christine Lind CEO, John Öhd CMO and Richard Bethell CSO will be hosting the conference.

To join the conference call, please dial:

Sweden +46 (0)8 566 426 62

Europe +44 20 3008 98 01

USA +1 855 7532 235

The webcast can also be accessed on the website: www.medivir.com

About disease modification in osteoarthritis

Osteoarthritis affects over 30 million adults in the US³, and as many as 240 million people worldwide. There are currently no disease-modifying therapies approved for the treatment of the disease and all approved osteoarthritis treatments affect only day to day symptoms and have no effect on the degenerative changes in the diseased joint⁴. In order to exert a disease modifying effect on osteoarthritis a prospective DMOAD needs to show efficacy on the degenerative changes seen in the joint in terms of bone and cartilage, as well as on clinical benefit.

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. It is being developed to slow or reverse the progressive degeneration of joints affected by osteoarthritis, and is therefore referred to as a Disease Modifying Osteoarthritis Drug (DMOAD). Since there are no DMOADs approved for use currently, the standard of care for osteoarthritis patients is based on changes in life style and the use of analgesics. The long-term use of analgesics by osteoarthritis patients is associated with an increased risk of side effects such as gastrointestinal bleeding and opioid dependency. DMOADs therefore represent a very large and attractive market opportunity. Medivir estimates that the US market alone is greater than USD 6 billion annually for a drug that impacts disease progression, even if its use was restricted to patient populations with moderate osteoarthritis in weight-bearing joints.

About the MIV-711 phase IIa studies

MIV-711-201 was a randomized, double-blind, placebo-controlled phase IIa clinical trial evaluating the safety and efficacy of 6 months of treatment with MIV-711 compared to placebo for the treatment of patients with moderate knee osteoarthritis. MIV-711-201 enrolled 244 patients. The primary endpoint was the change in patient-reported average knee pain. The change in joint bone area, assessed using magnetic resonance

imaging (MRI), was a key secondary endpoint as it has been shown to be a sensitive and precise measure of the long-term degenerative changes that take place in the structure of joints affected by osteoarthritis. Further information about MIV-711-201 can be found at www.clinicaltrials.gov with the identifier NCT02705625.

An open-label extension study, MIV-711-202, is assessing the safety, tolerability and efficacy of six additional months of treatment with MIV-711 in patients treated in the initial study for six months who showed evidence of response, and the safety, tolerability and efficacy of six months of treatment with MIV-711 in patients who received placebo in the initial study and whose osteoarthritis worsened. Further information about MIV-711-202 can be found at www.clinicaltrials.gov with the identifier NCT03037489.

The initial study and the extension study together provide an opportunity to assess the effect of 12 months of treatment on the structure of the diseased knee, and to assess the effect of 6 months of treatment on the structure of the diseased knee in a patient population whose symptoms may be progressing rapidly, and who may therefore derive greater benefit from treatment with a potential DMOAD.

For further information, please contact:

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Medivir AB is obliged to make this information public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact persons set out above, at 23.45 CET on 25 September 2017.

About Medivir

Medivir is a research-based pharmaceutical company with a focus on oncology. We have a leading competence within protease inhibitor design and nucleotide/nucleoside science and we are dedicated to develop innovative pharmaceuticals that meet great unmet medical needs. Medivir is listed on the Nasdaq Stockholm Mid Cap List.

1. Hunter D et al. Ann Rheum Dis. 2016 Sep;75(9):1607-14
2. Barr AJ et al, Rheumatology (Oxford). 2016 Sep;55(9):1585-93
3. <https://www.cdc.gov/arthritis/basics/osteoarthritis.htm>
4. https://www.oarsi.org/sites/default/files/docs/2016/oarsi_white_paper_oa_serious_disease_121416_1.pdf