Remetinostat phase II data demonstrate efficacy on skin lesions, reduction of itching and high tolerability in patients with early-stage MF-type CTCL

Stockholm, Sweden - Medivir AB (Nasdaq Stockholm: MVIR) today announces that phase II efficacy and safety data in patients with Mycosis Fungoides (MF) type early-stage Cutaneous T-cell Lymphoma (CTCL) demonstrated that remetinostat gel 1%, when applied topically twice daily, reduced the severity of CTCL skin lesions. Remetinostat also caused a clinically significant reduction in the severity of pruritus (itching) in those patients with clinically significant pruritus at the start of the study, and was highly tolerable with no systemic adverse effects.

These data were contained in an abstract published at the European Organization for Research and Treatment of Cancer (EORTC) Cutaneous Lymphoma Task Force meeting being held in London on October 13-15th. The details of the study are scheduled to be presented at the meeting by Dr Madeleine Duvic, Professor of Internal Medicine and Dermatology at the MD Anderson Cancer Center in Houston, Texas.

The remetinostat phase II trial enrolled 60 patients with stage IA-IIA MF, the predominant variant of CTCL, across five different clinical sites in the USA. Patients were randomized to receive one of remetinostat gel 0.5% twice daily, remetinostat gel 1% once daily, or remetinostat gel 1% twice daily, for up to 12 months.

The primary end-point of the study was the proportion of patients with either a complete or partial confirmed response to therapy, assessed using the Composite Assessment of Index Lesion Severity. The study showed a dose response with patients in the remetinostat gel 1% twice daily arm having the highest proportion of confirmed responses including 1 complete response. Based on an intent-to-treat analysis, confirmed response rates in patients were as follows:

Dose Arm	Number of Patients per Arm	Number of Patients with Confirmed Response (whereof complete response)	% of Patients with Confirmed Response
1% twice daily	20	8 (1)	40%
0.5% twice daily	20	5 (0)	25%
1% once daily	20	4 (0)	20%

As a secondary objective the effect of remetinostat gel on severity of pruritus was assessed monthly for the duration of the study using the visual analogue scale (VAS). Among patients with clinically significant pruritus at baseline, those who received remetinostat gel 1% twice daily had the highest proportion of patients achieving clinically significant reduction in pruritus from baseline. The proportions of patients who had confirmed, clinically significant, reductions in pruritus from baseline, defined as at least a 30mm reduction in the VAS score sustained for >4 weeks, were as follows:

Dose Arm	% Patients with Confirmed Clinically Significant Reduction in Pruritus from Baseline
1% twice daily	80%
0.5% twice daily	50%
1% once daily	37.5%

Remetinostat was generally well tolerated, with adverse events evenly distributed across the treatment arms. The most common adverse events were skin related and mostly grade 1-2. There were no signs of systemic

adverse effects related to remetinostat treatment, including those associated with systemic HDAC inhibitors. Most patients remained on study for the maximum possible duration and the median treatment time was 350 days.

"The beneficial effects of remetinostat on both CTCL lesions and the pruritus associated with early-stage CTCL that were observed in this clinical trial are highly encouraging, as was its safety profile" said John Öhd, Chief Medical Officer at Medivir AB. "Given the chronic nature of this cancer and the limited availability of safe, effective and convenient treatments, patients with early-stage CTCL are in need of new treatment options that effectively control the symptoms of the disease and that are safe to use over long periods for time. The results of the phase II study support the progression of remetinostat into a pivotal clinical program."

Based on the outcomes of the phase II study, Medivir expects to meet with regulatory authorities to discuss the design of a pivotal clinical program for remetinostat in MF-type CTCL.

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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 12.45 CET on 13 October 2017.

About Mycosis Fungoides (MF) and Cutaneous T-cell Lymphoma (CTCL)
Cutaneous T-cell Lymphoma (CTCL) is an orphan hematologic cancer that presents primarily in the skin. According to the National Cancer Institute, mycosis fungoides (MF) is the most common variant of CTCL. MF affects an estimated 15,000 to 20,000 people in the United States, with an estimated 1,500 new cases annually. Around 75% of affected patients are in early-stages (stages IA-IIA) where the disease is confined to the skin. Patients remain at these stages for extended periods and require long-term, skin-directed treatments for their disease. A small proportion of patients go on to develop cutaneous tumours or systemic disease, and these patients then require systemic anti-cancer therapy, which may include systemic histone deacetylase (HDAC) inhibitors. Pruritus, or itching, is a major symptom that adversely affects quality of life in many CTCL patients. Available topical drugs for early-stage CTCL are not always effective and tolerable, and sometimes worsen pruritus. Medivir estimates that the addressable market for an efficacious and tolerable treatment for early-stage CTCL in the US alone is approximately USD 900m annually.

About remetinostat
Remetinostat is a uniquely topical histone deacetylase (HDAC) inhibitor that Medivir is developing for the treatment of early-stage mycosis fungoides (MF) type CTCL. Remetinostat has been designed to be effective in the skin but degraded rapidly in the bloodstream to avoid the adverse effects associated with systemically administered HDAC inhibitors.

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.