press release

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FDA grants Glycostem's oNKord® Orphan Drug Designation for Multiple Myeloma

Designation will accelerate oNKord®'s market access for MM.

Together with Clinical Trial Approval for a pivotal phase I-IIa trial in AML, this is another important step forward in making Glycostem's allogenic Natural Killer Cell-based treatment accessible for cancer patients.

12th October 2020, Oss, the Netherlands – Glycostem Therapeutics, a leading clinical-stage company focused on the development of therapeutic off-the-shelf Natural Killer (NK) cells, announces it has received the FDA's Orphan Drug Designation (ODD) for treatment of Multiple Myeloma (MM) patients with its investigational product oNKord®. The designation will provide Glycostem with certain incentives, like eligibility for 7 years of market exclusivity and clear FDA guidance on specific aspects of development for rare diseases. These pave an accelerated path towards market access and treatment of patients suffering from this relatively rare form of cancer.

oNKord® is Glycostem's first-generation off-the-shelf Natural Killer (NK) cellular immunotherapy product. Over the coming months, AML patients will receive this form of treatment as part of a phase I-IIa (pivotal) trial in AML. A phase II trial for MM patients is expected to start in 2021. This makes Glycostem one of the frontrunners in this promising field of cellular immunotherapy.

"Since 2012 we have been pioneers in the field of developing and manufacturing off-the-shelf Natural Killer cell therapy products for cancer treatment. In 2020 we're entering a new and exciting phase," says Troels Jordansen, CEO of Glycostem. "It is great to experience that after receiving FDA and EMA ODD designation for AML, the FDA has also granted us this designation for MM. This allows us to accelerate oNKord®'s access to the US market and our ultimate ambition: curing cancer."

Multiple Myeloma (MM)

MM is the second most common blood cancer, accounting for 15% of blood cancers, and 2% of all cancers. In the US alone it affects more than 130,000 patients; approximately 32,000 Americans are diagnosed with MM each year. MM occurs in infection-fighting plasma cells (a type of white blood cell) found in the bone marrow. These cancerous cells multiply, produce an abnormal protein and push out other healthy blood cells from the bone marrow.

Orphan Drug Designation

The FDA grants orphan drug designation to drugs and biologics for the prevention, diagnosis, or treatment of diseases or conditions affecting fewer than 200,000 persons in the US. The designation allows

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manufacturers to qualify for various incentives, including exemption of FDA application fees), tax credits for qualified clinical trials and be eligible for 7 years of market exclusivity after regulatory approval.

About Glycostem

Netherlands-based Glycostem Therapeutics BV, a clinical stage biotech company, develops allogeneic cellular immunotherapy to treat several types of cancer. By harnessing the power of stem cell-derived Natural Killer (NK) cells, Glycostem's products are a safe alternative to CAR-T-cells. Glycostem's lead product, oNKord®, is manufactured from allogeneic raw material and is available off-the shelf. Thanks to its nine patent families, longstanding technical expertise and resources, as well as 'Orphan Drug Designation', Glycostem has secured a leadership position in the global NK-cell market.

oNKord® is produced in a closed system in Glycostem's state-of-the-art and GMP (Good Manufacturing Practice) licensed production facility in the Netherlands, from which it can be distributed globally. The production technology includes *ex vivo* generation of high numbers of NK-cells with a high degree of purity for clinical applications. oNKord® successfully passed phase I clinical trial (elderly and frail AML - Acute Myeloid Leukemia - patients), providing solid safety data and strong indication of clinical activity, including response on MRD (Minimal Residual Disease). Results indicate that oNKord® may be safely infused in AML patients.

Glycostem is furthermore developing a range of CAR-NK products in-house and in cooperation with global partners.

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