PRESS RELEASE

FDA grants Breakthrough Therapy Designation for Debiopharm's novel chemo-radio sensitizer Debio 1143 for front-line treatment of Head & Neck Cancer

- *There have been no newly approved therapies over the last 25 years for "high risk" locally advanced squamous cell carcinoma of the head and neck (LA-SCCHN) patients*
- *The FDA decision was based on the compelling magnitude of the clinically phase II findings in combination with chemo-radiotherapy presented in September 2019 at the ESMO congress (European Society for Medical Oncology)*
- *The Breakthrough Therapy Designation reaffirms that Debio 1143 has the potential to offer a significant benefit over the current standard of care in LA-SCCHN, responding to the high unmet need in this debilitating cancer type*
- *Debio 1143 has the potential to be the first-in-class inhibitor of apoptosis proteins (IAP) antagonist for the treatment of high-risk, locally advanced squamous cell carcinoma of the head and neck (LA-SCCHN)*

Lausanne, Switzerland – February 27, 2020 – Debiopharm (www.debiopharm.com), a Swiss-based, global biopharmaceutical company, today announced that the American Food and Drug Administration (FDA) has granted a Breakthrough Therapy Designation for Debio 1143, the most clinically advanced IAP antagonist, for the treatment of patients with confirmed diagnosis of previously untreated, unresectable locally advanced squamous cell carcinoma of the head and neck (LA-SCCHN) in combination with current standard of care, cisplatin-based concomitant standard fractionation chemoradiation therapy (CRT). Ongoing clinical investigations have been developed to evaluate the benefit of increasing tumor cell sensitivity to CRT, making the addition of Debio 1143 a highly promising strategy for radio-oncologists to improve treatment outcomes for high-risk LA-SCCHN.

This Breakthrough Therapy Designation is based on the clinically compelling phase II study results (NCT02022098) presented at the ESMO Congress 2019 in Barcelona, Spain. They revealed a very significant improvement of the primary endpoint locoregional control rate at 18 months after CRT (21% improvement vs. control arm) and a marked Progression-Free Survival (PFS) benefit vs. the CRT+placebo arm after a 2-year follow-up period (HR=0.37, p=0.007). In addition, the compound showed a predictable and manageable safety profile, that did not compromise the full delivery of standard CRT.

"Despite today's current standard of care, high-risk locally-advanced head and neck cancer remains an area of unmet medical need. This Breakthrough Therapy Designation will allow us to maximize the potential of Debio 1143 to become an innovative radio-chemo enhancing treatment for LA-SCCHN patients," **commented Angela Zubel, Chief Development Officer, Debiopharm.**

"This FDA assessment is a strong encouragement to expand investigations into other cancer types where the radio-sensitization effect of Debio 1143 could also provide further benefits over the current standard of care," **expressed Sergio Szyldergemajn, Medical Director of Oncology at Debiopharm.**

Breakthrough Therapy Designation is intended to expedite the clinical development and review of medicines showing substantial improvements in serious or life-threatening conditions so that patients can access innovative therapies as soon as possible.

About Head and Neck Cancer

Squamous cell carcinoma of the head and neck (SCCHN) is the 6th most common cancer type worldwide, with more than half of patients diagnosed with locally-advanced (LA) disease.[1-2] High-risk LA-SCCHN patients, including HPV negative oropharyngeal cancer (OPC) patients and heavy smokers, face a poor prognosis even with current standard of care (SOC) as more than half of them will relapse.[3-6] The consequences of LA-SCCHN have a heavy impact on quality of life and social interactions, affecting how patients look, talk, eat and breathe.[7-9] Additional therapies are needed to ensure better outcomes for patients facing this devastating condition.

About Debio 1143

Debio 1143 is a potential first-in-class oral antagonist of IAPs (inhibitor of apoptosis proteins), that sensitizes tumor cells to radio-chemo therapy by promoting programmed cell death and fostering anti-tumor immunity. The clinical benefit observed in *LA-SCCHN patients* suggests that the integration of Debio 1143 into widely used CRT regimens is a promising investigational approach over a broad range of cancer types. Currently poised to enter into a Phase III, pivotal trial later this year in combination with CRT in Head & Neck cancer, the compound is also being investigated along with immune checkpoint inhibitors (PD-1/PD-L1) in various solid tumors. Over 200 patients have been treated so far with Debio 1143 in various indications and lines of treatment, showing an adequate and consistent safety profile across studies.

Debiopharm's commitment to cancer patients

Debiopharm aims to develop innovative therapies that target high unmet medical needs in oncology. Bridging the gap between disruptive discovery products and real-world patient reach, we identify high-potential compounds for in-licensing, clinically demonstrate their safety and efficacy and then select large pharmaceutical commercialization partners to maximize patient access globally.
For more information, please visit www.debiopharm.com
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References

1. ESMO. Head & Neck Cancers: Essentials for Clinicians. 2017. p. 1–6. http://oncologypro.esmo.org/content/download/113133/1971849/file/2017-ESMO-Essentials-for-Clinicians-Head-Neck-Cancers-Chapter-1.pdf (accessed August 2019)
2. Perri F et al. Future Sci OA. 2018;5(1).
3. Ang KK et al. N Engl J Med 2010;363:24-35.
4. Marur S et al. Curr Opin Oncol. 2014;26(3):252–258
5. Magnes T et al. MEMO. 2017;10(4):220–223.
6. Du E et al. Laryngoscope. 2019.
7. Nelke K et al. Adv Clin Exp Med. 2014;23(6):1019–1027
8. Rettig EM et al. Cancer. 2016;122(12):1861–1870.
9. Hernández-Vila C Plast Aesthet Res. 2015;3:203-210