Volasertib, a potential new treatment for Rhabdomyosarcoma, receives Orphan Drug Designation from the U.S. FDA

BOSTON, MASSACHUSETTS, UNITED STATES, October 14, 2020 /EINPresswire.com/ -- Oncoheroes Biosciences, a biotech focused on advancing new therapies for childhood cancer, today announced that the U.S. Food and Drug Administration has granted Orphan Drug Designation (ODD) to volasertib for its use in treating pediatric rhabdomyosarcoma and other rare soft tissue sarcomas.

Volasertib receives FDA's Orphan Drug Designation

Key Points
- FDA awards Orphan Drug Designation to potential therapies addressing unmet needs of underserved patients with rare diseases.
- ODD may enable Oncoheroes to retain seven years of U.S. market exclusivity upon marketing approval and obtain partial tax credits for clinical trial expenditures.
- Volasertib previously received Rare Pediatric Disease Designation by the FDA, which may also provide substantial financial incentives to Oncoheroes with the related Priority Review Voucher program.

The U.S. FDA has programs to encourage companies to develop drugs for rare diseases, defined as diseases affecting 200,000 people in the U.S. at the time of designation. Since the Orphan Drug Act was signed into law in 1983, the FDA has granted Orphan Drug Designations (ODD) and later approved hundreds of drugs for rare diseases.

“All the ODD-related incentives will help us to accelerate the clinical development and commercialization of our first asset volasertib, an investigational treatment for rhabdomyosarcoma. Each potential new therapy for rhabdomyosarcoma gives fresh hope to the around 450 American families diagnosed each year with this type of childhood cancer,” stated Ricardo Garcia, Oncoheroes’ Founder, and CEO.

Under the Orphan Drug status, Oncoheroes will qualify for various development incentives,

including a tax credit on expenditures incurred in clinical studies, exemption from filing fees such as the user fee, a cost savings of around $2.9M in 2020, eligibility for a research grant awarded by the FDA, and, most importantly, seven years of U.S. market exclusivity upon approval. Being granted orphan designation does not modify the standard regulatory requirements to obtain marketing approval.

“The fact that volasertib was granted FDA’s Rare Pediatric Disease Designation last month, and now, Orphan Drug Designation speaks to the unmet medical need for better treatments for patients with rhabdomyosarcoma and the strength of the translational data package supporting volasertib for rhabdomyosarcoma,” stated Cesare Spadoni, Oncoheroes’ Founder and COO.
In September 2020, volasertib was also granted with the Rare Pediatric Disease Designation (RPDD), making Oncoheroes eligible for a fast track review and for a Priority Review Voucher (PRV) that is fully transferable to other sponsor companies and historically have had a selling price range of USD 67 to 350 million.

Around 450 new patients each year in the U.S. are diagnosed with rhabdomyosarcoma (RMS), the most common soft tissue sarcoma in children and young adults, representing around 3% of childhood cancers. It is an aggressive and highly malignant form of cancer that develops from skeletal muscle cells that have failed to fully differentiate. Despite efforts to improve treatment, survival rates have only improved in the medium or low-risk groups. In contrast, over the past 25 years, a threshold was reached with no improvement in survival for high-risk patients. Therefore, there is a clear unmet medical need to treat the most aggressive forms of this disease.

Volasertib is an inhibitor of Polo-like-kinase 1 (PLK1), an enzyme known to be involved in disease progression in a number of cancers. The compound was originally discovered and developed by Boehringer Ingelheim for the treatment of Acute Myeloid Leukemia, until the company decided to discontinue the compound for strategic reasons. Meanwhile, independent academic groups generated strong data in support of further development of volasertib for rhabdomyosarcoma and, possibly, a few other pediatric cancer indications. In 2019, Oncoheroes in-licensed volasertib from Boehringer Ingelheim to continue the clinical development of this drug candidate for the benefit of younger cancer patients.

Preclinical research in rhabdomyosarcoma

A number of publications highlight the potential of volasertib in rhabdomyosarcoma. High PLK1 expression has been associated with poor prognosis in a number of cancers, including rhabdomyosarcoma. It was shown that the drug may have a specific anti-cancer effect in this disease, which is driven by the PAX3-FOXO1 fusion protein in a large subset of patients. PAX3-FOXO1 is a challenging drug target. However, it was shown that PLK1 inhibition by volasertib reduces the stability of this fusion protein leading to its degradation and cancer growth inhibition in PDX models. Interestingly, in vivo data also point to a strong synergy between volasertib and vincristine, a drug already in the standard treatment protocol for rhabdomyosarcoma. Most of these data were generated by European laboratories that are part

of the Innovative Therapies for Children with Cancer (ITCC) consortium. Oncoheroes is planning to collaborate with ITCC for the clinical development of volasertib.

About Oncoheroes Biosciences Inc.

Oncoheroes is a ground-breaking biotech company exclusively focused on the discovery and development of better drugs for children and adolescents with cancer. Our vision is to deliver benefits to young cancer patients and create value in the process. The company is headquartered in Boston, US, with a discovery lab in Barcelona, Spain. Oncoheroes is actively looking for in-licensing opportunities in the pediatric oncology space while working to generate new proprietary assets for a number of pediatric cancer indications with high unmet medical needs.

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