FDA Grants Rare Pediatric Disease Designation (RPDD) to Volasertib for Rhabdomyosarcoma

BOSTON, MASSACHUSETTS, UNITED STATES, September 24, 2020 /EINPresswire.com/ -- Oncoheroes Biosciences, a biotech focused on advancing new therapies for childhood cancer, is pleased to announce that the United States Food and Drug Administration (FDA) has granted the designation of rare pediatric disease to volasertib, an investigational treatment for rhabdomyosarcoma.

Key Points

• Rare Pediatric Disease Designation qualifies Oncoheroes to receive fast track review, and a priority review voucher (PRV) at the time of marketing approval of volasertib.

• PRV holder can benefit from an expedited six-month review of a new drug application for any disease by the FDA.

• PRVs are transferable to other sponsor companies and historically have had a selling price range of USD 67 to 350 million.

The US FDA actively supports companies that develop drugs for rare diseases, defined as diseases affecting less than 200,000 Americans. One of the relevant programs created by the agency is for rare diseases primarily affecting individuals younger than 18 years old, called the Rare Pediatric Disease Designation (RPDD) which comes with the related priority review voucher opportunity.

“We are delighted that volasertib has been awarded with RPDD by the FDA. This recognition acknowledges the unmet medical need for better treatments for children and adolescents with rhabdomyosarcoma. We hope this big news will allow Oncoheroes to speed up the drug

development process of volasertib," stated Ricardo Garcia, Oncoheroes' Founder and CEO.

Upon drug approval, the RPDD may provide substantial financial incentives by making companies eligible for a Priority Review Voucher (PRV) that is fully transferable. The PRV grants accelerated FDA review of a drug candidate, for any indication, reducing the review period to 6 months and potentially gaining early market access. To date, 12 out of 25 PRVs received for pediatric indications have been sold for a cumulative sale price of USD 1.6 billion.

"Current rhabdomyosarcoma treatments are based on decades-old therapies and generally lack efficacy against the most aggressive subtypes of the disease, for which the 5-year survival rate is currently 20-30%. We are excited about upcoming clinical studies and we hope that volasertib could be a game-changer for rhabdomyosarcoma patients," explained Cesare Spadoni, PhD, Oncoheroes' Founder and COO.

Around 500 new patients each year in the US are diagnosed with rhabdomyosarcoma, an aggressive and highly malignant form of cancer (soft tissue sarcoma) that develops from skeletal muscle cells that have failed to fully differentiate. There is a clear unmet medical need for the treatment of 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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