FDA Grants Rare Pediatric Disease Designation (RPDD) to dovitinib for osteosarcoma

BOSTON, MASSACHUSETTS, USA, September 23, 2022 / EINPresswire.com/ -- Oncoheroes Biosciences, Inc. (“Oncoheroes”) is pleased to announce that the United States Food and Drug Administration (FDA) has granted the designation of rare pediatric disease to dovitinib, an investigational treatment for osteosarcoma.

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 dovitinib.
- The 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 have had a recent average selling price of $105 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.

Upon drug approval, the RPDD program 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, 18 out of 34 PRVs received for pediatric indications have been sold for a cumulative sale price of USD 2.3 billion.

Osteosarcoma (OS) is a rare disease and the most common cause of bone cancer in children and adolescents; OS is diagnosed in approximately 750-1,000 new individuals each year in the United States. About 450 are children or adolescents under the age of 20. The demonstration of the utility of chemotherapy for all patients in the early 1980s led to the current survival rate of > 65%, but this has not changed over the past three decades. The 5-year overall survival rates are approximately 20% for patients who develop metastatic disease. Additionally, of patients who experience disease progression or recurrence, less than 30% survive.

“We are delighted that dovitinib has been granted RPDD by the FDA. This is an acknowledgment of the urgent need for better treatments for children and adolescents with osteosarcoma. We hope this fantastic news can speed up the drug’s clinical development process,” stated Ricardo Garcia, Oncoheroes’ Co-Founder, and CEO.

Dovitinib is a pan-tyrosine kinase inhibitor targeting fibroblast growth factor receptor (FGFR), vascular endothelial growth factor receptor (VEGFR), and other receptor tyrosine kinases (RTKs). In January 2022, Oncoheroes signed an exclusive pediatric licensing agreement for dovitinib with Allarity Therapeutics, Inc. to continue the clinical development of this drug candidate for the benefit of younger cancer patients. Dovitinib will be developed together with Allarity’s DRP® companion diagnostics.

Several RTKs inhibited by dovitinib are involved in the growth, differentiation, and survival of different types of tumor cells as well as in tumor angiogenesis and development and maintenance of the tumor stroma microenvironment that is essential for proliferation of tumor cells and angiogenesis. Many RTKs must be inhibited simultaneously to generate a clinical response. Generally, the efficacy of tyrosine kinase inhibitors (TKIs) in the clinic depends on achieving a wide therapeutic index (vs off-tumor/on-target toxicity). Resistance can quickly occur to single TKI through mutations and therefore resistance is less likely to quickly develop with TKIs as these hit multiple targets simultaneously. Given dovitinib’s multi-target mechanism of action, the drug’s utility in pediatric osteosarcoma is promising.

“A major gap in the field, and one that continues to challenge the development of clinically effective TKIs in osteosarcoma, is the lack of reliable biomarkers”, said Cesare Spadoni, Oncoheroes’ Co-Founder, and COO. “Our partnership with Allarity brings the potential added value of further validating Allarity’s DRP® companion diagnostics for the selection of patients that are most likely to respond to dovitinib”.

About Oncoheroes Biosciences

Oncoheroes Biosciences 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.

For more information please visit: oncoheroes.com

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