Novartis receives FDA Orphan Drug Designation for NIS793 in pancreatic cancer

Jul 27, 2021

Novartis today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) for NIS793 in combination with standard of care chemotherapy for the treatment of pancreatic cancer. NIS793 is a potential first in class novel antibody specific for Transforming Growth Factor Beta (TGFβ), which is known to have an important role in metastatic pancreatic ductal carcinoma (mPDAC) and other solid tumors.

- Pancreatic cancer has one of the lowest survival rates of any cancer, with typically late detection and poor outcomes with standard of care treatment. The 5-year overall survival rate is approximately 11% in the US[1], with few novel approaches advancing in the clinic and targeted and immunotherapy agents having shown limited activity.
- NIS793 is a fully human anti-TGF-β IgG2 monoclonal antibody designed to inhibit the TGF-β pathway in tumor cells and to modulate the tumor microenvironment. In preclinical models, inhibiting TGFβ can reduce fibrosis characteristic of pancreatic and other solid tumor types, as well as enhance the response to chemotherapy and immunotherapy.
- NIS793 has shown proof of mechanism and acceptable safety profile in a first in-human trial in patients with advanced solid tumors[2] and is in development for pancreatic cancer and other solid tumor types. A Phase II study is ongoing, and a Phase III trial in 1L mPDAC is planned to start enrolling patients later this year.

An ODD grants special status to a drug being developed to treat a rare disease or condition, and provides companies certain benefits to encourage the continued development of medicines that bring novel solutions to patients with these diseases.[3]

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1. https://prod1.novartis.com/news/novartis-receives-fda-orphan-drug-designation-nis793-pancreatic-cancer