AstraZeneca and Merck’s Selumetinib Would Become the First Medicine Indicated for the Treatment of Certain Pediatric Patients with NF1 Plexiform Neurofibromas if Approved

KENILWORTH, N.J.--(BUSINESS WIRE)--AstraZeneca and Merck (NYSE:MRK), known as MSD outside the United States and Canada, today announced that the U.S. Food and Drug Administration (FDA) has accepted a New Drug Application (NDA) and granted priority review for the MEK 1/2 inhibitor selumetinib as a potential new medicine for pediatric patients aged three years and older with neurofibromatosis type 1 (NF1) and symptomatic, inoperable plexiform neurofibromas (PNs).

This is the first acceptance of a regulatory submission for an oral MEK 1/2 monotherapy for patients with NF1, a rare and incurable genetic condition. A Prescription Drug User Fee Act (PDUFA) date is set for the second quarter of 2020.

This regulatory submission was based on positive results from the National Cancer Institute (NCI) Cancer Therapy Evaluation Program (CTEP)-sponsored SPRINT Phase 2 Stratum 1 trial. An objective response rate (ORR) was achieved in 66% of pediatric patients with NF1 and symptomatic, inoperable PNs (n=33/50 patients) when treated with selumetinib as a twice-daily oral monotherapy. ORR was defined as the percentage of patients with a confirmed complete or partial response of ≥ 20% tumor volume reduction.

Selumetinib was granted U.S. FDA Breakthrough Therapy Designation for this population in April of 2019, U.S. FDA Orphan Drug Designation in February of 2018, EU Orphan Drug Designation by the European Medicines Agency in August 2018, and Swissmedic Orphan Drug Status in December 2018. AstraZeneca and Merck have a strategic collaboration agreement to co-develop and co-commercialize selumetinib globally.

About SPRINT
The SPRINT trial is a U.S. NCI CTEP-sponsored Phase 1/2 trial. The Phase 1 trial was designed to identify the optimal Phase 2 dosing regimen, and the results were published in the New England Journal of Medicine.

About Selumetinib
Selumetinib is an investigational MEK 1/2 inhibitor. It is designed to inhibit the MEK enzyme in the RAS/MAPK pathway, a cell-signaling pathway, associated with cancer cell growth and proliferation in a number of different tumor types.

About Neurofibromatosis Type 1 (NF1)
NF1 is an incurable genetic condition that affects one in every 3,000 to 4,000 individuals. It is caused by a spontaneous or inherited mutation in the NF1 gene and is associated with many symptoms, including soft lumps on and under the skin (cutaneous neurofibromas), skin pigmentation (so-called ‘cafe au lait’ spots) and, in 30-50% of patients, tumors develop on the nerve sheaths (plexiform neurofibromas). These plexiform neurofibromas can cause clinical issues such as pain, motor dysfunction, airway dysfunction, bowel/bladder dysfunction and disfigurement as well as having the potential to transform into malignant peripheral nerve sheath tumors (MPNST).

People with NF1 may experience a number of complications such as learning difficulties, visual impairment, twisting and curvature of the spine, high blood pressure, and epilepsy. NF1 also increases a person’s risk of developing other cancers, including malignant brain tumors, MPNST and leukemia. Symptoms begin during early childhood, with varying degrees of severity, and can reduce life expectancy by up to 15 years.

About the AstraZeneca and Merck Strategic Oncology Collaboration
In July 2017, AstraZeneca and Merck, known as MSD outside the United States and Canada, announced a global strategic oncology collaboration to co-develop and co-commercialize certain oncology products, including investigational selumetinib, a MEK inhibitor. Working together, the companies will develop selumetinib in combination with other potential new medicines and as monotherapy. Independently, the companies will develop selumetinib in combination with their respective PD-L1 and
PD-1 medicines.

**Merck's Focus on Cancer**

Our goal is to translate breakthrough science into innovative oncology medicines to help people with cancer worldwide. At Merck, the potential to bring new hope to people with cancer drives our purpose and supporting accessibility to our cancer medicines is our commitment. As part of our focus on cancer, Merck is committed to exploring the potential of immuno-oncology with one of the largest development programs in the industry across more than 30 tumor types. We also continue to strengthen our portfolio through strategic acquisitions and are prioritizing the development of several promising oncology candidates with the potential to improve the treatment of advanced cancers. For more information about our oncology clinical trials, visit [www.merck.com/clinicaltrials](http://www.merck.com/clinicaltrials).

**About Merck**

For more than a century, Merck, a leading global biopharmaceutical company known as MSD outside of the United States and Canada, has been inventing for life, bringing forward medicines and vaccines for many of the world's most challenging diseases. Through our prescription medicines, vaccines, biologic therapies and animal health products, we work with customers and operate in more than 140 countries to deliver innovative health solutions. We also demonstrate our commitment to increasing access to health care through far-reaching policies, programs and partnerships. Today, Merck continues to be at the forefront of research to advance the prevention and treatment of diseases that threaten people and communities around the world - including cancer, cardio-metabolic diseases, emerging animal diseases, Alzheimer's disease and infectious diseases including HIV and Ebola. For more information, visit [www.merck.com](http://www.merck.com) and connect with us on Twitter, Facebook, Instagram, YouTube and LinkedIn.

**Forward-Looking Statement of Merck & Co., Inc., Kenilworth, N.J., USA**

This news release of Merck & Co., Inc., Kenilworth, N.J., USA (the “company”) includes “forward-looking statements” within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. These statements are based upon the current beliefs and expectations of the company’s management and are subject to significant risks and uncertainties. There can be no guarantees with respect to pipeline products that the products will receive the necessary regulatory approvals or that they will prove to be commercially successful. If underlying assumptions prove inaccurate or risks or uncertainties materialize, actual results may differ materially from those set forth in the forward-looking statements.

Risks and uncertainties include but are not limited to, general industry conditions and competition; general economic factors, including interest rate and currency exchange rate fluctuations; the impact of pharmaceutical industry regulation and health care legislation in the United States and internationally; global trends toward health care cost containment; technological advances, new products and patents attained by competitors; challenges inherent in new product development, including obtaining regulatory approval; the company’s ability to accurately predict future market conditions; manufacturing difficulties or delays; financial instability of international economies and sovereign risk; dependence on the effectiveness of the company’s patents and other protections for innovative products; and the exposure to litigation, including patent litigation, and/or regulatory actions.

The company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise. Additional factors that could cause results to differ materially from those described in the forward-looking statements can be found in the company’s 2018 Annual Report on Form 10-K and the company’s other filings with the Securities and Exchange Commission (SEC) available at the SEC’s Internet site ([www.sec.gov](http://www.sec.gov)).

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