ASTRAZENECA PLC
Form 6-K
April 01, 2019

FORM 6-K

SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

Report of Foreign Issuer

Pursuant to Rule 13a-16 or 15d-16 of
the Securities Exchange Act of 1934

For the month of April 2019

Commission File Number: 001-11960

AstraZeneca PLC

1 Francis Crick Avenue
Cambridge Biomedical Campus
Cambridge CB2 0AA
United Kingdom

Indicate by check mark whether the registrant files or will file annual reports under cover of Form 20-F or Form 40-F.

Form 20-F X Form 40-F __

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(1):

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(7): _____

Indicate by check mark whether the registrant by furnishing the information contained in this Form is also thereby furnishing the information to the Commission pursuant to Rule 12g3-2(b) under the Securities Exchange Act of 1934.

Yes __ No X

If “Yes” is marked, indicate below the file number assigned to the Registrant in connection with Rule 12g3-2(b): 82-_____

AstraZeneca PLC

INDEX TO EXHIBITS

1.
Selumetinib gets Breakthrough Therapy Designation

1 April 2019 07:00 BST

Selumetinib granted US Breakthrough Therapy
Designation in neurofibromatosis type 1

Designation based on Phase II SPRINT trial in
paediatric patients with NF1 plexiform neurofibromas

Selumetinib is a MEK 1/2 Inhibitor being co-developed by AstraZeneca and MSD

AstraZeneca and MSD, Inc., Kenilworth, NJ, US (MSD: known as Merck & Co., Inc. inside the US and Canada) today announced that the US Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation (BTD) for the MEK 1/2 inhibitor and potential new medicine selumetinib.

This designation is for the treatment of paediatric patients aged three years and older with neurofibromatosis type 1 (NF1) symptomatic and/or progressive, inoperable plexiform neurofibromas (PN), a rare, incurable genetic condition.

José Baselga, Executive Vice President, Research and Development, Oncology, said: "Selumetinib shows promise in the treatment of NF1-related plexiform neurofibromas, a rare and debilitating disease with no approved medications to date. The Breakthrough Therapy Designation acknowledges the significant unmet need of these patients and the potential benefit of selumetinib in this setting."

Roy Baynes, Senior Vice President and Head of Global Clinical Development, Chief Medical Officer, at MSD Research Laboratories, said: "This new designation validates our ongoing development of selumetinib. As a result of this, selumetinib has the potential to receive expedited regulatory review and we hope to bring this medicine to patients as soon as possible."

The BTD is based on Phase II data from the SPRINT trial, testing selumetinib as an oral monotherapy in paediatric patients, aged three years or older with inoperable NF1-related PN. The results of the trial were presented by the National Cancer Institute (NCI) at the 2018 American Society of Clinical Oncology Annual Meeting.

This is the ninth BTD that AstraZeneca has received from the FDA since 2014. BTD is designed to expedite the development and regulatory review of medicines that are intended to treat a serious condition and that have shown encouraging early clinical results, which may demonstrate substantial improvement on a clinically-significant endpoint over available medicines.

Selumetinib was granted Orphan Drug Designation for the treatment of NF1 by the US FDA in February 2018 and the European Medicines Agency in August 2018.

Selumetinib is a MEK 1/2 inhibitor and potential new medicine licensed by AstraZeneca from Array BioPharma Inc. in 2003. AstraZeneca and MSD entered a co-development and co-commercialisation agreement for selumetinib in 2017.

The NF1 gene provides instructions for making a protein called neurofibromin, which negatively regulates the RAS/MAPK pathway, helping to control cell growth, differentiation and survival. Mutations in the NF1gene may result in dysregulations in RAS/RAF/MEK/ERK signalling, which can cause cells to grow, divide and copy themselves in an uncontrolled manner, and may result in tumour growth. Selumetinib inhibits the MEK enzyme in this pathway, potentially leading to inhibition of tumour growth.

Selumetinib is being assessed as a monotherapy and in combination with other treatments in ongoing trials.

About SPRINT
The SPRINT trial is a US Cancer Therapy Evaluation Program (CTEP) NCI-sponsored Phase I/II trial. The Phase I trial was designed to identify the optimal Phase II dosing regimen, and the results were published in the New England Journal of Medicine.1

About NF1
Neurofibromatosis type 1 (NF1) is an incurable genetic condition that affects one in 3,000 to 4,000 individuals.2,3 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 20-50% of patients, tumours 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 tumours (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 tumours, MPNST and leukaemia. Symptoms begin during early childhood, with varying degrees of severity, and can reduce life expectancy by up to 15 years.4

About the AstraZeneca and MSD Strategic Oncology Collaboration
In July 2017, AstraZeneca and Merck & Co., Inc., Kenilworth, NJ, US, known as MSD outside the United States and Canada, announced a global strategic oncology collaboration to co-develop and co-commercialise Lynparza (olaparib), the world's first PARP inhibitor and potential new medicine selumetinib, a MEK inhibitor, for multiple cancer types. Working together, the companies will develop Lynparza and selumetinib in combination with other potential new medicines and as monotherapies. Independently, the companies will develop Lynparza and selumetinib in combination with their respective PD-L1 and PD-1 medicines.

About AstraZeneca in Oncology
AstraZeneca has a deep-rooted heritage in Oncology and offers a quickly-growing portfolio of new medicines that has the potential to transform patients' lives and the Company's future. With at least six new medicines to be launched between 2014 and 2020, and a broad pipeline of small molecules and biologics in development, we are committed to advance New Oncology as one of AstraZeneca's five Growth Platforms focused on lung, ovarian, breast and blood cancers. In addition to our core capabilities, we actively pursue innovative partnerships and investments that accelerate the delivery of our strategy, as illustrated by our investment in Acerta Pharma in haematology.

By harnessing the power of four scientific platforms - Immuno-Oncology, Tumour Drivers and Resistance, DNA Damage Response and Antibody Drug Conjugates - and by championing the development of personalised

combinations, AstraZeneca has the vision to redefine cancer treatment and one day eliminate cancer as a cause of death.

About AstraZeneca
AstraZeneca is a global, science-led biopharmaceutical company that focuses on the discovery, development and commercialisation of prescription medicines, primarily for the treatment of diseases in three therapy areas - Oncology, Cardiovascular, Renal & Metabolism and Respiratory. AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide. For more information, please visit astrazeneca.com and follow us on Twitter @AstraZeneca.

Media Relations		
Gonzalo Viña	UK/Global	+44 203 749 5916
Rob Skelding	UK/Global	+44 203 749 5821
Matt Kent	UK/Global	+44 203 749 5906
Jennifer Hursit	UK/Global	+44 203 749 5762
Christina Malmberg Hägerstrand	Sweden	+46 8 552 53 106
Michele Meixell	US	+1 302 885 2677

Investor Relations		
Thomas Kudsk Larsen		+44 203 749 5712
Henry Wheeler	Oncology	+44 203 749 5797
Christer Gruvris	BioPharma - Cardiovascular; Metabolism	+44 203 749 5711
Nick Stone	BioPharma - Respiratory; Renal	+44 203 749 5716
Josie Afolabi	Other	+44 203 749 5631
Craig Marks	Finance; Fixed Income	+44 7881 615 764
Jennifer Kretzmann	Retail Investors; Corporate Access	+44 203 749 5824
US toll-free		+1 866 381 72 77

Adrian Kemp
Company Secretary
AstraZeneca PLC

References

1 Dombi E, et al. Activity of Selumetinib in Neurofibromatosis Type 1-Related Plexiform Neurofibromas. N Engl J Med. 2016; 375:2550-2560.
2 NHS Choices. Neurofibromatosis Type 1. Available at https://www.nhs.uk/conditions/neurofibromatosis-type-1/. Accessed March 2019.
3 Johnson KJ, et al. Development of an International Internet-based Neurofibromatosis Type 1 Patient Registry. Contemporary Clinical Trials. 2013;34:305-311.
4 Evans DGR, et al. Reduced Life Expectancy Seen in Hereditary Diseases Which Predispose to Early-Onset Tumors. Appl Clin Genet. 2013; 6:53-61

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

AstraZeneca PLC

Date: 01 April 2019

By: /s/ Adrian Kemp
Name: Adrian Kemp
Title: Company Secretary