

ASTRAZENECA PLC  
Form 6-K  
February 15, 2018

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 February 2018

Commission File Number: 001-11960

AstraZeneca PLC

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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  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

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

AstraZeneca PLC

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Selumetinib in NF1 gets FDA orphan drug status

15 February 2018 07:00 GMT

## SELUMETINIB GRANTED ORPHAN DRUG DESIGNATION BY THE US FDA FOR NEUROFIBROMATOSIS TYPE 1

AstraZeneca and Merck & Co., Inc., Kenilworth, NJ, US (known as MSD outside the US and Canada) today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) for selumetinib, a MEK 1/2 inhibitor, for the treatment of neurofibromatosis type 1 (NF1).

NF1 is an incurable genetic condition that affects one in 3,000 births,[i] with highly-variable symptoms, including cutaneous (skin), neurological (nervous system) and orthopaedic (skeletal) manifestations. NF1 can cause secondary complications including learning difficulties, visual impairment, pain, disfigurement, twisting and curvature of the spine, high blood pressure and epilepsy. Plexiform neurofibromas (PNs) are a neurological manifestation of NF1 and arise from nerve fascicles that tend to grow along the length of the nerve. PNs occur in approximately 20-50% of NF1 patients causing pain, motor dysfunction and disfigurement.[ii]

Sean Bohan, Executive Vice President, Global Medicines Development and Chief Medical Officer, at AstraZeneca, said: "Neurofibromatosis type 1 is a devastating condition that can lead to life-threatening complications. There is no known cure for neurofibromatosis and there are limited treatment options to manage symptoms."

Roy Baynes, Senior Vice President and Head of Global Clinical Development, Chief Medical Officer, at MSD Research Laboratories, said: "We're looking forward to working with our colleagues at AstraZeneca to develop selumetinib and understand how it may benefit patients with NF1."

The potential benefit of selumetinib in NF1 is being explored in the US National Cancer Institute-sponsored Phase I/II SPRINT trial in paediatric patients with symptomatic NF1-related PNs. Phase II trial results are expected later in 2018.

The FDA's ODD programme provides orphan status to medicines that are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the US.

In addition to NF1, selumetinib is being investigated in the Phase III ASTRA trial of patients who are diagnosed with differentiated thyroid cancer (DTC) following surgery and treatment with radioactive iodine. Selumetinib was granted ODD by the US FDA for the adjuvant treatment of stage III/IV DTC in 2016. It is also being explored as a monotherapy and in combination with other treatments in Phase I trials.

### About neurofibromatosis type 1 (NF1)

NF1 is caused by a spontaneous or inherited mutation in the NF1 gene. The disease is associated with many symptoms, including soft lumps on and under the skin (subcutaneous neurofibromas), skin pigmentation (cafe au lait spots) and, in 20-50% of patients, tumours on the nerve sheaths (plexiform neurofibromas). These plexiform neurofibromas can cause morbidities such as pain, motor dysfunction and disfigurement. Patients with NF1 may experience a number of other complications such as learning difficulties, visual impairment, twisting and curvature of the spine, high blood pressure, and epilepsy. People with NF1 also have an increased risk of developing other cancers, including malignant brain and peripheral nerve sheath tumours, and leukaemia. Symptoms begin during early childhood, with varying degrees of severity, and can reduce life expectancy by up to 15 years.[iii]

#### About selumetinib

Selumetinib is an investigational MEK 1/2 inhibitor licensed by AstraZeneca from Array BioPharma Inc. in 2003.

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 NF1 gene 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.

#### 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. The collaboration is based on increasing evidence that PARP and MEK inhibitors can be combined with PD-L1/PD-1 inhibitors for a range of tumour types. Working together, the companies will develop Lynparza and selumetinib in combination with other potential new medicines and as a monotherapy. 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 & Metabolic Diseases and Respiratory. The Company also is selectively active in the areas of autoimmunity, neuroscience and infection. AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide.

For more information, please visit [www.astrazeneca.com](http://www.astrazeneca.com) and follow us on Twitter @AstraZeneca.

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Adrian Kemp  
Company Secretary, AstraZeneca PLC

[i] Ghalayani P, et al. Neurofibromatosis Type I (von Recklinghausen's Disease): A Family Case Report and Literature Review. Dent Res J. 2012;9(4): 483-488.

[ii] Dombi E, et al. Activity of Selumetinib in Neurofibromatosis Type 1-Related Plexiform Neurofibromas. N Engl J Med. 2016; 375:2550-2560.

[iii] 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: 15 February 2018

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