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## AstraZeneca granted Breakthrough Therapy Designation for rare genetic condition treatment

AstraZeneca PLC's (LON:AZN) potential treatment for a rare genetic condition called neurofibromatosis type 1 (NF1) has been granted breakthrough therapy designation (BTD) by US regulators.

The move from the US Food and Drug Administration effectively fast-tracks the regulatory review of the medicine, called selumetinib, which Astra is working on with fellow pharma giant Merck & Co Inc (NYSE:MRK).

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NF1 is a rare genetic condition that causes tumours to grow along a person's nerves which can lead to various skin issues and learning difficulties, as well as problems with the bones, eyes and nervous system.

People with NF1 also have a much higher rate of cancer and cardiovascular disease.

The BTD is based on data from a Phase II monotherapy trial last summer which showed that selumetinib can reduce pain and motor impairment.

"Selumetinib shows promise in the treatment of NF1-related plexiform neurofibromas, a rare and debilitating disease with no approved medications to date," said Astra's oncology R&D boss José Baselga.

"The Breakthrough Therapy Designation acknowledges the significant unmet need of these patients and the potential benefit of selumetinib in this setting."

**Price:** 6380p

**Market Cap:** £83691.76M

### 1 Year Share Price Graph



### Share Information

**Code:** AZN

**Listing:** LSE

**52 week High Low**  
6,634.00p 5,312.00p

**Sector:** Pharma & Biotech

**Website:** [www.astrazeneca.com](http://www.astrazeneca.com)

### Company Synopsis:

*AstraZeneca is one of the world's leading pharmaceutical companies, with a broad range of medicines designed to fight disease. AstraZeneca has over 67,000 employees and operates in over 100 countries with growing presence in important emerging markets.*

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