GLAXOSMITHKLINE PLC
Form 6-K
April 01, 2016

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 period ending April 2016

GlaxoSmithKline plc
(Name of registrant)

980 Great West Road, Brentford, Middlesex, TW8 9GS
(Address of principal executive offices)

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

Form 20-F x Form 40-F

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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

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Issued: Friday 1 April London UK - LSE Announcement

GSK receives positive CHMP opinion in Europe for StrimvelisTM, the first gene therapy to treat very rare disease, ADA-SCID

GlaxoSmithKline (LSE/NYSE: GSK) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA), in conjunction with the Committee for Advanced Therapies (CAT), has issued a positive opinion recommending marketing authorisation for Strimvelis to treat patients with a very rare disease called ADA-SCID (severe combined immunodeficiency due to adenosine deaminase deficiency). The medicine is a stem cell gene therapy created for an individual patient from their own cells which is intended to correct the root cause of the disease. If approved by the European Commission, the medicine - currently known as GSK2696273 (autologous CD34+ cells transduced to express ADA) - will be commercialised under the brand name Strimvelis, for the treatment of patients with ADA-SCID for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.

The gene therapy for the treatment of ADA-SCID was originally developed by Ospedale San Raffaele (OSR) and Fondazione Telethon (Telethon), through their joint San Raffaele Telethon Institute for Gene Therapy (SR-TIGET) and was taken forward by GSK through a strategic collaboration formed in 2010 between GSK, OSR and Telethon. Within the partnership GSK, working with the biotechnology company MolMed S.p.A, has applied its expertise in product development to optimise, standardise and characterise a manufacturing process that was previously only suitable for clinical trials into one that has been demonstrated to be robust and suitable for commercial supply.

Patrick Vallance, President, R&D, GSK said, "We welcome this opinion from the CHMP which is an important step towards making Strimvelis available to the children living with this incredibly rare and fatal condition. Going forward, we hope to apply this gene therapy platform technology across other diseases, enabling many more patients to benefit from this innovative treatment approach."

Martin Andrews, Head of the Rare Disease Unit at GSK said, "This positive opinion is a major milestone in GSK's commitment to the development of innovative, transformative medicines. If approved, Strimvelis will become the first corrective ex-vivo gene therapy for children to achieve regulatory approval anywhere in the world. With our shared mission and complementary expertise we believe this collaboration will continue to deliver much needed new medicines for patients with rare genetic diseases."

Until the EU Commission decision, Strimvelis will remain an investigational gene therapy which is not approved for use anywhere in the world.

About ADA-SCID
ADA-SCID is a very rare disorder caused by a faulty gene inherited from both parents. This faulty gene stops the production of an essential protein called adenosine deaminase (ADA), which is required for the production of lymphocytes (a type of white blood cell). Children born with ADA-SCID do not develop a healthy immune system so cannot fight off everyday infections, which results in severe and life-threatening illness. Without prompt treatment, the disorder often proves fatal within the child's first year of life. ADA-SCID is estimated to occur in approximately 15 patients per year in Europe.

About Strimvelis
Replacing faulty hematopoietic (or 'blood') stem cells has been carried out in the form of bone marrow transplants for more than 40 years. However, these transplants rely on cells taken from an immune matched, or closely related, immune matched donor. As immune (or HLA) matching is never perfect, immune incompatibility can cause rejection

called graft versus host disease (GvHD). GvHD is a common life threatening side effect that requires management with potent immunosuppressant medicines which in themselves increase the risk of infection and other complications.

Strimvelis is only administered once and does not rely on a third-party donor so there is no risk of GvHD. The patient's own bone marrow cells are removed, and a vector is used to insert a normal copy of the ADA gene into the cells. This step is known as transduction. The gene-corrected cells are then re-introduced to the patient via an intravenous infusion, after which some of the cells home back to the bone marrow. In order to improve the engraftment of the gene-modified cells in the patient's bone marrow, patients are also pre-treated with low dose chemotherapy.

Pivotal clinical study (n=12) and overall population treated within the primary data package (n=18)
A 100% survival rate at 3 years post-treatment with Strimvelis (primary endpoint) has been observed for all 12 children in the pivotal study, with 92% having interventional-free survival (i.e. did not require enzyme replacement therapy for a period of >3 months post-treatment or hematopoietic stem cell transplantation). All 18 children treated with Strimvelis who contributed data to the marketing authorisation application are alive today with a median follow-up duration of approximately 7 years, with the first of these having received this gene therapy over 13 years ago. Interventional-free survival within the evaluable population (n=17) was 82%.

Overall the safety findings are in line with those expected in children with ADA-SCID who have undergone treatment with low-dose chemotherapy and who are undergoing immune recovery. A significant reduction in severe infections has been documented and no leukaemic events have been observed to date.

GSK - one of the world's leading research-based pharmaceutical and healthcare companies - is committed to improving the quality of human life by enabling people to do more, feel better and live longer. For further information please visit www.gsk.com.

Fondazione Telethon - Telethon is a major biomedical charity in Italy whose mission is to advance biomedical research towards the cure of rare genetic diseases. For further information, visit www.telethon.it/en/

Ospedale San Raffaele - Ospedale San Raffaele is a clinical-research-university hospital established in 1971 to provide international-level specialised care for the most complex and difficult health conditions. Since 2012 it is part of the San Donato Hospital Group, the leading hospital group in Italy. The group includes 18 hospitals with a total of more than 5000 beds, 4000 physicians and treats 4 million patients per year. For further information, visit: www.hsr.it

San Raffaele Telethon Institute for Gene Therapy (SR-TIGET) - Based in Milan, Italy, the San Raffaele-Telethon Institute for Gene Therapy (SR-TIGET) is a joint venture between the Ospedale San Raffaele and Telethon. SR-TIGET was established in 1995 to perform research on gene transfer and cell transplantation and translate its results into clinical applications of gene and cell therapies for different genetic diseases. For further information, visit http://www.tiget.it/.

Strimvelis is a trade mark of the GSK group of companies.

GSK enquiries:			
UK Media enquiries:	Fiona McMillan	+44 (0) 20 8047 5502	(London)
	Claire Brough	+44 (0) 20 8047 5502	(London)
US Media enquiries:	Sarah Alspach	+1 202 715 1048	(Washington, DC)
	Sarah Spencer	+1 215 751 3335	(Philadelphia)

Analyst/Investor enquiries:	Ziba Shamsi	+44 (0) 20 8047 5543	(London)
	Tom Curry	+ 1 215 751 5419	(Philadelphia)
	Gary Davies	+44 (0) 20 8047 5503	(London)
	James Dodwell	+44 (0) 20 8047 2406	(London)
	Jeff McLaughlin	+1 215 751 7002	(Philadelphia)

Cautionary statement regarding forward-looking statements
GSK cautions investors that any forward-looking statements or projections made by GSK, including those made in this announcement, are subject to risks and uncertainties that may cause actual results to differ materially from those projected. Such factors include, but are not limited to, those described under Item 3.D 'Risk factors' in the company's Annual Report on Form 20-F for 2015.

Registered in England & Wales:
No. 3888792

Registered Office:
980 Great West Road
Brentford, Middlesex
TW8 9GS

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 authorised.

GlaxoSmithKline plc
(Registrant)

Date: April 01, 2016

By: VICTORIA WHYTE

Victoria Whyte
Authorised Signatory for and on
behalf of GlaxoSmithKline plc