GLAXOSMITHKLINE PLC
Form 6-K
May 27, 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 27 May 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 27 May London UK - LSE Announcement

StrimvelisTM receives European marketing authorisation to treat very rare disease, ADA-SCID

GSK, Fondazione Telethon and Ospedale San Raffaele gain approval to provide life-saving gene therapy to patients

GlaxoSmithKline (GSK), Fondazione Telethon (Telethon) and Ospedale San Raffaele (OSR) today announced that the European Commission has approved Strimvelis, the first ex-vivo stem cell gene therapy to treat patients with a very rare disease called ADA-SCID (Severe Combined Immunodeficiency due to Adenosine Deaminase deficiency). A child born with ADA-SCID does not have a healthy, fully-functioning immune system and as a consequence, is unable to fight off everyday infections. Strimvelis (autologous CD34+ cells transduced to express ADA) is the first corrective gene therapy for children to be awarded regulatory approval anywhere in the world. It is indicated for the treatment of patients with ADA-SCID for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.

ADA-SCID affects an estimated 15 children per year in Europe and following today's approval, patients with the condition who are referred for treatment will be able to receive the gene therapy at Ospedale San Raffaele in Milan.

Martin Andrews, Head of the Rare Disease Unit, GSK said: "Today's approval is the result of many years' work with our collaborators in Milan and is the next step towards bringing life-changing treatment to patients with ADA-SCID and their families. This is the start of a new chapter in the treatment of rare genetic diseases and we hope that this therapeutic approach could also be used to help patients with other rare diseases in the future."

The marketing authorisation decision was based on data collected from 18 children treated with Strimvelis. A 100% survival rate at three years post-treatment with Strimvelis was observed for all children in the pivotal study (n=12) and every child receiving the treatment who contributed to the marketing authorisation data package is alive today (n=18), with a median follow-up duration of approximately seven years. Full results of the analysis have recently been published in BLOOD1.

Professor Alessandro Aiuti, Clinical Research Coordinator at San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), a joint research collaboration between OSR and Telethon, said: "We are delighted with today's news, which marks the culmination of more than 20 years of research and development at Tiget. This innovative, individualised treatment approach uses a patient's own gene modified stem cells to correct the root cause of the disease. It has been gratifying for all of us to see patients affected by this severe immune deficiency growing over the years, being able to play with other children and going to school. Working alongside GSK, we can now make Strimvelis available to ADA-SCID patients and transform the lives of children who so desperately need it."

Nicola Bedin, CEO of Ospedale San Raffaele, said: "This great achievement would not have been possible without the effective collaboration between OSR, Telethon and GSK, which has brought together years of scientific research, first-class medical practice and expertise in product development. Going forward we hope to build on our shared mission to develop and deliver more much-needed new medicines to patients with rare diseases."

Francesca Pasinelli, General Manager of Fondazione Telethon, said: "This is a memorable day, not only for us, but overall for the people we work for: with Strimvelis we can keep our promise to patients. We can say that we have pioneered a model whereby the charity organisation acts not only as a funding agency, but plays a primary role in managing the development of research to ensure that each step of the process leads to the ultimate goal, which is to provide accessible therapy to patients."

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

Strimvelis is only administered once and does not rely on a third-party donor, so there is no risk of immune incompatibility causing rejection (graft versus host disease), which is a common side effect of bone marrow transplant treatment. With Strimvelis, 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.

Within the primary data package which formed the basis of marketing authorisation, a 100% survival rate at 3 years post-treatment with Strimvelis (primary endpoint) was observed for all 12 children in the pivotal study, with 92% having intervention-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.Intervention-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.

About the GSK / Telethon / OSR collaboration

The gene therapy for the treatment of ADA-SCID was originally developed in Milan 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 collaboration 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.

Important Safety Information for Strimvelis in the European Union

Overall the safety findings in the study were in line with those expected in children with ADA-SCID who have undergone treatment with low-dose chemotherapy and who are undergoing immune recovery. Adverse events were reported for all 18 patients; the most frequently reported being usual childhood infections including upper respiratory tract infection, gastroenteritis and rhinitis. Of the 39 serious adverse events which were reported post-GT, 62% were infections, with the most common being device-related infections, for example, from the central venous catheter (CVC) used during the treatment. Five patients reported SAEs due to CVC infection, three due to gastroenteritis and three due to pneumonia. A number of patients also experienced neurologic, CNS or hearing impairments which continued post-GT. 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 - Fondazione Telethon is a major biomedical charity in Italy whose mission is to advance biomedical research towards the cure of rare genetic diseases. Throughout its 26 years of activity, the Telethon Foundation has invested over €450 million in funding over 2,500 projects to study 470 diseases, involving more than 1,500 researchers. For further information, visit www.telethon.it/en

Ospedale San Raffaele - Ospedale San Raffaele (OSR) 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 OSR is part of Gruppo Ospedaliero San Donato, the leading hospital group in Italy. The hospital is a multi-specialty centre with over 50 clinical specialties and has over 1,300 beds. Research at OSR focuses on integrating basic, translational and clinical activities to provide the most advanced care to our patients. 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.

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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.

References

1 Cicalese, MP et al. Update on the safety and efficacy of retroviral gene therapy for immunodeficiency due to adenosine deaminase deficiency. BLOOD. DOI 10.1182/blood-2016-01-688226 http://www.bloodjournal.org/content/early/2016/04/29/blood-2016-01-688226 Last accessed May 2016

Registered in England & Wales:
No. 3888792

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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: May 27, 2016

By: VICTORIA WHYTE

Victoria Whyte
Authorised Signatory for and on
behalf of GlaxoSmithKline plc