



Pfizer Receives Breakthrough Therapy Designation from FDA for PF-06651600, an oral JAK3 Inhibitor, for the Treatment of Patients with Alopecia Areata

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Phase II data accepted for late-breaker news session at 2018 EADV Congress

NEW YORK--(BUSINESS WIRE)--Pfizer Inc. (NYSE:PFE) today announced its investigational oral Janus kinase 3 (JAK3) inhibitor PF-06651600 received Breakthrough Therapy designation from the U.S. Food and Drug Administration (FDA) for the treatment of patients with alopecia areata, a chronic autoimmune skin disease that causes hair loss on the scalp, face, or body.^{1,2}

The Breakthrough Therapy designation for alopecia areata was supported by positive results from a Phase 2 study, which will be presented during the late-breaking news session at the 27th European Academy of Dermatology and Venerology (EADV) Congress in Paris on September 15, 2018. Currently, there are no FDA-approved treatments for alopecia areata, which impacts millions of people worldwide and is often associated with profound psychological consequences.^{1,2}

“We are encouraged by this Breakthrough Therapy designation as it underscores the potential of our JAK3 inhibitor to address a critical unmet need,” said Michael Corbo, Chief Development Officer, Inflammation & Immunology, Pfizer Global Product Development. “We are continuing to work closely with the FDA on the development process with the goal of bringing this potential new treatment to patients living with alopecia areata as

soon as possible.”

Breakthrough Therapy designation was initiated as part of the Food and Drug Administration Safety and Innovation Act (FDASIA) signed in 2012. As defined by the FDA, a Breakthrough Therapy is a drug intended to be used alone or in combination with one or more other drugs to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. If a drug is designated as a Breakthrough Therapy, the FDA will expedite the development and review of such drug.³

Pfizer is also working with the European Medicines Agency (EMA) on the clinical development program for PF-06651600.

About Alopecia Areata

Alopecia areata is an autoimmune disease, characterized by hair loss, often patchy, on the scalp, face, or body.^{1,2} People suffering from alopecia areata experience symptoms when immune cells attack healthy hair follicles, causing the hair to fall out, often starting with smooth, round patches.^{1,2} The mean age of onset is between 25 and 35, but it can also impact children and adolescents, and is seen in both sexes and all ethnicities.^{1,2} More than half of patients with alopecia areata experience poor health-related quality of life and, as a result, the condition may lead to serious psychological consequences, including high levels of depression and anxiety.¹

About PF-06651600 and Pfizer’s Kinase Inhibitor Leadership

The JAK pathways are believed to play an important role in inflammatory processes as they are involved in signaling for over 50 cytokines and growth factors, many of which drive immune-mediated conditions.⁴ JAK inhibition offers the potential for new advanced treatment options for these conditions through unique and targeted selectivity.⁵

“Pfizer strives to continue moving the JAK science forward with development of multiple JAKis with unique selectivity profiles that are being evaluated in clinical trials. These inhibitors have the potential of meeting significant unmet needs in multiple inflammatory conditions,” said Michael Vincent, Chief Scientific Officer, Pfizer Inflammation & Immunology.

PF-06651600 is an oral small molecule that selectively inhibits Janus kinase (JAK) 3.5 PF-06651600 is also under investigation for the treatment of rheumatoid arthritis, Crohn's disease and ulcerative colitis.

Pfizer has established a leading kinase research capability with multiple unique kinase inhibitor therapies in development. As a pioneer in JAK science, the Company is continuing to advance several investigational programs for molecules with novel selectivity profiles, which, if approved, could potentially deliver transformative therapies for patients. In addition to PF-06651600, Pfizer has the following kinase inhibitors in trials across multiple indications:

PF-04965842: A selective JAK1 inhibitor in Phase 3 clinical trials for the treatment of atopic dermatitis(AD)⁶; PF-04965842 received Breakthrough Therapy designation from the FDA for the treatment of patients with moderate-to-severe AD PF-06700841: A tyrosine kinase 2(TYK2)/JAK1 inhibitor under investigation for the treatment of psoriasis, Crohn's disease, ulcerative colitis and alopecia areata PF-06650833: An interleukin-1 receptor associated kinase 4 (IRAK4) inhibitor under investigation for the treatment of rheumatoid arthritis PF-06826647: A TYK2 inhibitor under investigation for the treatment of psoriasis and inflammatory bowel disease (IBD)

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At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at www.pfizer.com. In addition, to learn more, please visit us on www.pfizer.com and follow us on Twitter at @Pfizer and @Pfizer_News, LinkedIn, YouTube and like us on Facebook at [Facebook.com/Pfizer](https://www.facebook.com/Pfizer).

DISCLOSURE NOTICE: The information contained in this release is as of September 5, 2018. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about PF-06651600 and Pfizer's ongoing investigational programs in kinase inhibitor therapies, including their potential benefits, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical trial commencement and completion dates and regulatory submission dates, as well as the possibility of unfavorable clinical trial results, including unfavorable new clinical data and additional analyses of existing data; risks associated with preliminary data; the risk that clinical trial data are subject to differing interpretations, and, even when we view data as sufficient to support the safety and/or effectiveness of a product candidate, regulatory authorities may not share our views and may require additional data or may deny approval altogether; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when drug applications may be filed in any jurisdictions for any potential indication for PF-06651600 or any other investigational kinase inhibitor therapies; whether and when any such applications may be approved by regulatory authorities, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted, and, if approved, whether PF-06651600 or any such other investigational kinase inhibitor therapies will be commercially successful; decisions by regulatory authorities regarding labeling, safety and other matters that could affect the availability or commercial potential of PF-06651600 or any other investigational kinase inhibitor therapies; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2017 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and www.pfizer.com.

1 Villasante Fricke AC, Miteva M. Epidemiology and burden of alopecia areata: a systematic review. *Clinical, Cosmetic and Investigational Dermatology*. 2015;8:397-403. doi:10.2147/CCID.S53985. 2 Pratt CH, King LE, Messenger AG, Christiano AM, Sundberg JP. Alopecia areata. *Nature reviews Disease primers*. 2017;3:17011. doi:10.1038/nrdp.2017.11. 3 Food and Drug Administration Fact Sheet Breakthrough Therapies at

<https://www.fda.gov/RegulatoryInformation/LawsEnforcedbyFDA/SignificantAmendmentstotheFD>

accessed on January 25, 2018 4 Banerjee, S., Biehl, A., Gadina, M. et al. JAK-STAT Signaling as a Target for Inflammatory and Autoimmune Diseases: Current and Future Prospects. *Drugs*. 2017;77: 521. <https://doi.org/10.1007/s40265-017-5> 5 Telliez JB, Dowty ME, Wang L, Jussif J, Lin T, Li L, et al. Discovery of a JAK3-selective inhibitor: functional differentiation of JAK3-selective inhibition over pan-JAK or JAK1-selective inhibition. *ACS Chem Biol*. 2016;11(12):3442-51. doi:10.1021/acscchembio.6b00677. 6 *J Med Chem*. 2018 Feb 8;61(3):1130-1152. doi: 10.1021/acs.jmedchem.7b01598. Epub 2018 Jan 23.

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