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PIERRE FABRE PHARMACEUTICALS STATEMENT REGARDING RECEIPT OF COMPLETE RESPONSE LETTER FOR TABELECLEUCEL BIOLOGICS LICENSE APPLICATION FROM THE U.S. FOOD AND DRUG ADMINISTRATION

SECAUCUS, N.J., January 12, 2026 /PRNewswire/ -- On January 9, 2026, Pierre Fabre Pharmaceuticals, Inc. received a Complete Response Letter (CRL) from the U.S. Food and Drug Administration (FDA) stating that the Agency is unable to approve the tabelecleucel Biologics License Application (BLA) in its present form.

We are surprised and deeply disappointed by the FDA's decision, particularly given the urgent and life-threatening unmet medical need faced by patients with Epstein-Barr virus–positive post-transplant lymphoproliferative disease (EBV+ PTLD) after failure of standard-of-care therapy. These patients have no FDA-approved treatment options and a life expectancy often measured in weeks to months.

The BLA was resubmitted following clear alignment with the FDA on the acceptability of the resubmission criteria and fulfillment of the conditions outlined in the January 15, 2025, CRL, which identified a single GMP-related deficiency and raised no concerns regarding safety, efficacy, or trial design. Upon acceptance of the resubmission in July 2025, the FDA granted tabelecleucel accelerated approval status.

In the new CRL, despite acknowledging that the GMP issue had been resolved and raising no safety concerns, the FDA stated that it no longer considers the previously accepted single-arm ALLELE study to be adequate to support accelerated approval and requested a new study. This represents a significant and unexpected change in position, and one that is contrary to extensive dialogue with the Agency over more than five years.

We are concerned that this decision may have far-reaching consequences for the development of rare disease treatments, effectively creating barriers for generating clinical evidence within a unique patient population with ultra-rare conditions thereby significantly delaying—or preventing altogether— patient access to urgently needed therapies.

We firmly believe that tabelecleucel represents an important treatment advance for patients with EBV+ PTLD and that the totality of data supports its efficacy and safety. We intend to engage with the FDA to urgently pursue a path forward, in collaboration with Atara Biotherapeutics (Nasdaq: ATRA) and our clinical and patient partners, to enable timely accelerated approval of tabelecleucel. We continue to be committed to making tabelecleucel available to patients through our Expanded Access Program.

Approval and real-world use of tabelecleucel over several years in multiple countries outside the United States further support its clinical value. We remain fully committed to securing approval of this critical treatment option for U.S. patients and the physicians who care for them.

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About Pierre Fabre Pharmaceuticals and Pierre Fabre Laboratories
The mission of Pierre Fabre Pharmaceuticals (PFP) is to deliver breakthrough therapies in oncology and rare diseases to patient populations with high unmet needs and limited treatment options. Our belief is that every time we care for a single person, we make the whole world better.

PFP is the US pharmaceutical subsidiary of Pierre Fabre Laboratories, a foundation-owned company with seven decades of impact. Pierre Fabre Laboratories is a global healthcare company, established in 43 countries, with over 10,000 employees, and with products distributed in 120 territories across the globe.

The Pierre Fabre Laboratories foundation ownership enhances the ability of the company to create long-term value for patients. Partnerships and acquisitions drive its innovative precision treatment pipeline and are enabled by the unique corporate structure.

Building on the legacy of Pierre Fabre Laboratories, innovation is the life blood of PFP and patient experience drives everything the company does. PFP aspires to design and develop therapeutic solutions inspired by patients and healthcare professionals; draw on science and nature as perpetual sources of inspiration; develop long-term partnerships with researchers and innovators worldwide; and place pharmaceutical ethics and climate transition at the heart of our action.

Pierre Fabre Pharmaceuticals has therapies in development for Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD), NRAS-mutant melanoma, non-small cell lung cancer with mutation or amplification of MET, and X-Linked Hypohidrotic Ectodermal Dysplasia (XLHED). Pierre Fabre Pharmaceuticals is headquartered in Secaucus, NJ.

For more information, visit www.pierrefabrepharmaceuticals.com, www.pierre-fabre.com, @Pierre Fabre Oncology

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