

February 26, 2021



## **Fortress Biotech Joins Global Movement to Raise Awareness for Rare Diseases and Supports Rare Disease Day®**

NEW YORK, Feb. 26, 2021 (GLOBE NEWSWIRE) -- Fortress Biotech, Inc. (NASDAQ: FBIO) ("Fortress"), an innovative revenue-generating company focused on acquiring, developing and commercializing or monetizing promising biopharmaceutical products and product candidates cost-effectively, today announced its support for Rare Disease Day® on February 28, an annual awareness day dedicated to elevating public understanding of rare diseases and calling attention to the special challenges people living with rare diseases face.

"Fortress Biotech stands with over 25 million Americans living with a rare disease and is a proud sponsor of the National Organization for Rare Disorders' (NORD®) 2021 Rare Disease Day activities. In collaboration with our partner companies, we are advancing innovative treatments for underserved rare diseases including AL amyloidosis, Menkes disease and X-linked severe combined immunodeficiency ("XSCID"), also known as bubble boy disease," said Lindsay A. Rosenwald, M.D., Chairman, President and Chief Executive Officer. "On this Rare Disease Day and every day, we are grateful to the patients participating in our clinical trials and their families for their commitment to helping others. We look forward to bringing therapies to patients with rare diseases who currently have no FDA-approved treatment options."

Fortress and its partner companies have multiple rare disease clinical programs underway including:

- The Cardiac Amyloid Reaching for Extended Survival (CARES) Phase 3 clinical program in AL amyloidosis, a rare systemic disorder caused by an abnormality of plasma cells in the bone marrow. Enrollment is ongoing in two parallel Phase 3 studies – one in patients with Mayo stage IIIa disease and one in patients with Mayo stage IIIb disease. (ClinicalTrials.gov Identifier: [NCT04512235](#) and [NCT04504825](#))
- A topline efficacy analysis was reported for Menkes disease, a rare X-linked recessive pediatric disease caused by gene mutations of copper transporter ATP7A that is often lethal if left untreated. A rolling submission of a New Drug Application ("NDA") to the U.S. Food and Drug Administration is anticipated to begin in the third quarter of 2021 and be completed in the fourth quarter of 2021. (ClinicalTrials.gov Identifier: [NCT04074512](#))

- Two pivotal Phase 2 clinical trials are anticipated to commence this year for XSCID, a rare genetic disorder that is characterized by the absence or lack of function of key immune cells, resulting in a severely compromised immune system and death by one year of age if untreated. The first trial, RESTORE, will enroll newly diagnosed XSCID patients  $\leq 2$  years of age and the second trial, SUSTAIN, plans to enroll XSCID patients who have received a prior hematopoietic stem cell transplant and now have waning immunity.
- A multicenter, pivotal Phase 1/2 clinical trial is enrolling patients with BPDCN, a rare, incurable and aggressive hematologic malignancy of the bone marrow and blood that can affect other organs such as the lymph nodes, spleen, central nervous system and skin. BPDCN has a median survival of less than 2 years, and there is no standard of care for relapsed or refractory disease. (ClinicalTrials.gov Identifier: [NCT04109482](https://clinicaltrials.gov/ct2/show/study/NCT04109482))
- A Phase 1 single-center, two-arm clinical trial is currently enrolling patients with leptomeningeal brain tumors (e.g., glioblastoma, ependymoma or medulloblastoma), a form of metastatic brain cancer that is difficult to treat. Leptomeningeal disease occurs when a cancer spreads to the membranes lining the brain and spinal cord and to the cerebrospinal fluid. (ClinicalTrials.gov Identifier: [NCT04661384](https://clinicaltrials.gov/ct2/show/study/NCT04661384))

In addition to its rare disease clinical programs, Fortress is a campaign supporter of NORD® and will also support “Light the Night for Amyloidosis” taking place during Amyloidosis Awareness Month in March. This campaign drives awareness about amyloidosis by encouraging participants to light up their entryways with red bulbs during the month.

According to the National Institutes of Health, a disease is rare if it affects fewer than 200,000 people in the United States. There are more than 7,000 rare diseases currently identified and approximately 90% of them are still without FDA-approved medical treatments.

### **About Rare Disease Day**

Rare Disease Day takes place every year on the last day of February—the rarest date on the calendar—to underscore the nature of rare diseases and what patients face. It was established in Europe in 2008 by EURORDIS, the organization representing rare disease patients in Europe, and is now observed in more than 80 nations. Rare Disease Day is sponsored in the U.S. by the National Organization for Rare Disorders (NORD®), the leading independent, nonprofit organization committed to the identification, treatment, and cure of rare diseases. The core message of NORD’s “Show Your Stripes” campaign is to wear stripes on Rare Disease Day to raise awareness and show support for those living with rare diseases. For more information about Rare Disease Day in the US or “Show Your Stripes®,” go to [rarediseaseday.us](https://rarediseaseday.us). For information about global activities, go to [rarediseaseday.org](https://rarediseaseday.org). To search for information about rare diseases, visit NORD’s website, [rarediseases.org](https://rarediseases.org).

### **About Fortress Biotech**

Fortress Biotech, Inc. (“Fortress”) is an innovative biopharmaceutical company that was ranked in Deloitte’s 2019 and 2020 Technology Fast 500™, annual rankings of the fastest-growing North American companies in the technology, media, telecommunications, life sciences and energy tech sectors, based on percentages of fiscal year revenue growth over three-year periods. Fortress is focused on acquiring, developing and commercializing high-

potential marketed and development-stage drugs and drug candidates. The company has five marketed prescription pharmaceutical products and over 25 programs in development at Fortress, at its majority-owned and majority-controlled partners and at partners it founded and in which it holds significant minority ownership positions. Such product candidates span six large-market areas, including oncology, rare diseases and gene therapy, which allow it to create value for shareholders. Fortress advances its diversified pipeline through a streamlined operating structure that fosters efficient drug development. The Fortress model is driven by a world-class business development team that is focused on leveraging its significant biopharmaceutical industry expertise to further expand the company's portfolio of product opportunities. Fortress has established partnerships with some of the world's leading academic research institutions and biopharmaceutical companies to maximize each opportunity to its full potential, including Alexion Pharmaceuticals, Inc., AstraZeneca, City of Hope, Fred Hutchinson Cancer Research Center, St. Jude Children's Research Hospital and Nationwide Children's Hospital. For more information, visit [www.fortressbiotech.com](http://www.fortressbiotech.com).

### **Forward-Looking Statements**

This press release may contain "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, as amended. As used below and throughout this press release, the words "we", "us" and "our" may refer to Fortress individually or together with one or more partner companies, as dictated by context. Such statements include, but are not limited to, any statements relating to our growth strategy and product development programs and any other statements that are not historical facts. Forward-looking statements are based on management's current expectations and are subject to risks and uncertainties that could negatively affect our business, operating results, financial condition and stock price. Factors that could cause actual results to differ materially from those currently anticipated include: risks relating to our growth strategy; our ability to obtain, perform under and maintain financing and strategic agreements and relationships; risks relating to the results of research and development activities; uncertainties relating to preclinical and clinical testing; risks relating to the timing of starting and completing clinical trials; our dependence on third-party suppliers; risks relating to the COVID-19 outbreak and its potential impact on our employees' and consultants' ability to complete work in a timely manner and on our ability to obtain additional financing on favorable terms or at all; our ability to attract, integrate and retain key personnel; the early stage of products under development; our need for substantial additional funds; government regulation; patent and intellectual property matters; competition; as well as other risks described in our SEC filings. We expressly disclaim any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in our expectations or any changes in events, conditions or circumstances on which any such statement is based, except as may be required by law, and we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995. The information contained herein is intended to be reviewed in its totality, and any stipulations, conditions or provisos that apply to a given piece of information in one part of this press release should be read as applying *mutatis mutandis* to every other instance of such information appearing herein.

### **Company Contacts:**

Jaclyn Jaffe and William Begien  
Fortress Biotech, Inc.  
(781) 652-4500

[ir@fortressbiotech.com](mailto:ir@fortressbiotech.com)

**Investor Relations Contact:**

Daniel Ferry  
LifeSci Advisors, LLC  
(617) 430-7576  
[daniel@lifesciadvisors.com](mailto:daniel@lifesciadvisors.com)

**Media Relations Contact:**

Tony Plohoros  
6 Degrees  
(908) 591-2839  
[tplohoros@6degreespr.com](mailto:tplohoros@6degreespr.com)



Source: Fortress Biotech, Inc.