Inhibrx Receives FDA Orphan-Drug Designation for INBRX-101 for the Treatment of Alpha-1 Antitrypsin Deficiency

- Interim results reported in October 2021 from the Phase 1 study showed a favorable safety and tolerability profile with no drug-related severe or serious adverse events at doses up to and including 120 mg/kg single dose and 80 mg/kg multi-dose administered intravenously, or IV.
- Data from the single ascending dose cohorts revealed the potential to achieve normal AAT levels with monthly dosing. Data from the first multiple ascending dose cohort at 40 mg/kg IV every three weeks showed the expected accumulation of functional AAT levels with observed trough levels exceeding those achieved by the current standard of care.
- Topline data expected to be announced from Part 2 of the Phase 1 trial in Q2 2022, with plans for an End of Phase 1 meeting with FDA to follow

SAN DIEGO, March 3, 2022 /PRNewswire/ -- Inhibrx, Inc. (Nasdaq: INBX), a biotechnology company with four clinical programs in development and a strong emerging pipeline, today announced that the U.S. Food and Drug Administration, or FDA, has granted orphan-drug designation for INBRX-101 for the treatment of alpha-1 antitrypsin deficiency, or AATD.

"We believe this designation from the FDA acknowledges the significant unmet need for novel therapeutic options in a patient community where the standard of care has not seen an improvement in many decades," said Inhibrx Chief Executive Officer, Mark Lappe. "We believe INBRX-101 can greatly improve the quality of life for AAT patients and look forward to working closely with regulators, key opinion leaders, patients and their families to achieve this."

The FDA's Office of Orphan Products Development grants orphan designation status to drugs and biologics that are intended for the safe and effective treatment, diagnosis or prevention of rare diseases, or conditions that affect fewer than 200,000 people in the U.S. Orphan-drug designation provides certain benefits, including financial incentives to support clinical development and the potential for up to seven years of market exclusivity in the U.S. upon regulatory approval.

About AATD and INBRX-101

Alpha-1 antitrypsin deficiency, or AATD, is an inherited orphan disease affecting an estimated 100,000 patients in the United States. AATD is characterized by deficient levels of the AAT protein, which causes loss of lung tissue and function and decreased life expectancy. Plasma-derived AAT is the current standard of care but does not maintain patients in the normal AAT range, and requires frequent and inconvenient once-weekly IV dosing, while relying on plasma collection practices that might not be sustainable.

INBRX-101 is a precisely engineered recombinant human AAT-Fc fusion protein designed to safely achieve and maintain levels of AAT found in healthy individuals with a favorable safety profile and the potential for once-monthly dosing.

In October 2021, the Company announced interim functional PK data from 21 AATD patients in the Phase 1 study. Dose related increases in maximal and total INBRX-101 exposure occurred across the entirety of the tested single ascending dose range of 10 to 120 mg/kg. Data from the first multiple ascending dose cohort of INBRX-101 at 40 mg/kg IV every three weeks showed the expected accumulation of functional AAT levels with observed trough levels exceeding the goal of the current standard augmentation therapy with plasma-derived AAT.

Interim safety data from 24 patients with AATD showed no drug-related severe or serious adverse events at doses up to and including 120 mg/kg single dose and 80 mg/kg multiple dose. Drug-related adverse events were predominantly mild with a few moderate events, and all were transient and reversible. No signs of neutralizing anti-drug antibodies have been observed.

About Inhibrx, Inc.

Inhibrx is a clinical-stage biotechnology company focused on developing a broad pipeline of novel biologic therapeutic candidates in oncology and orphan diseases. Inhibrx utilizes diverse methods of protein engineering to address the specific requirements of complex target and disease biology, including its proprietary sdAb

platform. Inhibrx has collaborations with 2seventy bio (formerly bluebird bio), Bristol-Myers Squibb and Chiesi, among others. For more information, please visit www.inhibrx.com.

Forward-Looking Statements

Inhibrx cautions you that statements contained in this press release regarding matters that are not historical facts are forward-looking statements. These statements are based on Inhibrx's current beliefs and expectations. These forward-looking statements include, but are not limited to, statements regarding: Inhibrx's and its investigators' judgments and beliefs regarding the strength of Inhibrx's pipeline, any future potential or observed to date safety and efficacy of its therapeutic candidate, INBRX-101, including potential dosing intervals, discussions with and beliefs regarding future action by the FDA and meetings with the FDA, statements and beliefs regarding the clinical development of INBRX-101, potential benefits of the orphan drug-designation and any presumption of positive results from Phase 1 clinical trials. Actual results may differ from those set forth in this press release due to the risks and uncertainties inherent in Inhibrx's business, including, without limitation, risks and uncertainties regarding: the initiation, timing, progress and results of its preclinical studies and clinical trials, and its research and development programs; its ability to advance therapeutic candidates into, and successfully complete, clinical trials; its interpretation of initial, interim or preliminary data from its clinical trials, including interpretations regarding disease control and disease response; the timing or likelihood of regulatory filings and approvals; the successful commercialization of its therapeutic candidates, if approved; the pricing, coverage and reimbursement of its therapeutic candidates, if approved; its ability to utilize its technology platform to generate and advance additional therapeutic candidates; the implementation of its business model and strategic plans for its business and therapeutic candidates; its ability to successfully manufacture therapeutic candidates for clinical trials and commercial use, if approved; its ability to contract with third-party suppliers and manufacturers and their ability to perform adequately; the scope of protection it is able to establish and maintain for intellectual property rights covering its therapeutic candidates; its ability to enter into strategic partnerships and the potential benefits of these partnerships; its estimates regarding expenses, capital requirements and needs for additional financing and financial performance; its expectations regarding the impact of the COVID-19 pandemic on its business; and other risks described from time to time in the "Risk Factors" section of its filings with the U.S. Securities and Exchange Commission, or the SEC, including those described in its Annual Report on Form 10-K for the year ended December 31, 2021 as filed with the SEC on February 28, 2022, as well as its Quarterly Reports on Form 10-Q, and supplemented from time to time by its Current Reports on Form 8-K. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and Inhibrx undertakes no obligation to update these statements to reflect events that occur or circumstances that exist after the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, which is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. This press release contains estimates and other statistical data made by independent parties and by Inhibrx. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates.

Investor and Media Contact:
Kelly Deck, CFO
kelly@inhibrx.com
858-795-4260

SOURCE Inhibrx, Inc.
