Fibrocell Awarded $1.4 Million FDA Orphan Grant for FCX-007 for Treatment of Recessive Dystrophic Epidermolysis Bullosa

September 25, 2018

EXTON, Pa., Sept. 25, 2018 (GLOBE NEWSWIRE) -- Fibrocell Science, Inc. (NASDAQ: FCSC), a gene therapy company focused on transformational autologous cell-based therapies for skin and connective tissue diseases, today announced that the U.S. Food and Drug Administration's (FDA) Office of Orphan Products Development (OOPD) has awarded a $1.4 million clinical trial research grant for Fibrocell's continued clinical development of FCX-007, the Company's gene therapy candidate for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a devastating, rare skin blistering disease with high mortality.

"We are delighted with the FDA's recognition to support the ongoing progress of our clinical trials of FCX-007," said John Maslowski, President and Chief Executive Officer of Fibrocell. "With no FDA approved therapies available, this grant further validates the significant opportunity of FCX-007's potential to relieve the pain and suffering from the debilitating, chronic blisters and open wounds of RDEB and offer hope to patients and their families."

Fibrocell's $1.4 million grant, which will be distributed over the next four years, was awarded by the FDA through the OOPD's Orphan Products Clinical Trials Grants Program. This program supports the clinical development of products for use in rare diseases or conditions for which "no current therapy exists or where the proposed product will be superior to the existing therapy." FDA stated in a press release for these awards that "[g]rant applications were reviewed and evaluated for scientific and technical merit by more than 100 rare disease experts, which included representatives from academia, the National Institutes of Health and the FDA."

FCX-007 is currently being evaluated in the Phase 2 portion of a Phase 1/2 clinical trial for the treatment of RDEB. Six patients ages seven and older are targeted to be treated with FCX-007 in the Phase 2 portion of the clinical trial. Fibrocell expects to report an interim data analysis for FCX-007 and provide a clinical trial update from Phase 1 patients and available data from Phase 2 patients in the first quarter of 2019.

The FDA has granted Orphan Drug Designation for the treatment of dystrophic epidermolysis bullosa, including RDEB, Rare Pediatric Disease Designation for the treatment of RDEB and Fast Track Designation for the treatment of RDEB to FCX-007.

Fibrocell is developing FCX-007 in collaboration with Precigen, Inc., a wholly owned subsidiary of Intrexon Corporation (NASDAQ: XON), a leader in synthetic biology. Fibrocell plans to manufacture FCX-007 at its cGMP cell manufacturing facility located in Exton, Pennsylvania.

About FCX-007

FCX-007 is Fibrocell's clinical stage, gene therapy product candidate for the treatment of RDEB, a congenital and progressive orphan skin disease caused by the deficiency of the protein type VII collagen (COL7). FCX-007 is a genetically-modified autologous fibroblast that encodes the gene for COL7 and is being developed in collaboration with Precigen, Inc. By genetically modifying autologous fibroblasts *ex vivo* to produce COL7, culturing them and then treating wounds locally via injection, FCX-007 offers the potential to address the underlying cause of the disease by providing high levels of COL7 directly to the affected areas while avoiding systemic distribution.

About the Phase 1/2 Clinical Trial

The primary objective of this open-label clinical trial is to evaluate the safety of FCX-007 in RDEB patients. Additionally, the trial is assessing wound healing and pharmacology at 4, 12, 25 and 52 weeks post-administration. Six patients ages seven and older are targeted to be treated with FCX-007 in the Phase 2 portion of the trial. To learn more about the clinical trial, please visit www.clinicaltrials.gov and search the identifier NCT02810951.

About Fibrocell

Fibrocell is an autologous cell and gene therapy company translating personalized biologics into medical breakthroughs for diseases affecting the skin and connective tissue. Fibrocell's most advanced product candidate, FCX-007, is the subject of a Phase 1/2 clinical trial for the treatment of RDEB. Fibrocell is also developing FCX-013, the Company's clinical stage candidate for the treatment of moderate to severe localized scleroderma. Fibrocell's gene therapy portfolio is being developed in collaboration with Precigen, Inc., a wholly owned subsidiary of Intrexon Corporation (NASDAQ: XON), a leader in synthetic biology. For more information, visit www.fibrocell.com or follow Fibrocell on Twitter at @Fibrocell.

Trademarks

Fibrocell®, the Fibrocell logo, and Fibrocell Science® are trademarks of Fibrocell Science, Inc. and/or its affiliates. All other names may be trademarks of their respective owners.

Forward-Looking Statements

This press release contains, and our officers and representatives may from time to time make, statements that are "forward-looking statements" within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. All statements that are not historical facts are hereby identified as forward-looking statements for this purpose and include, among others, statements relating to: Fibrocell's expectations regarding the timing and clinical development of FCX-007; the potential advantages of FCX-007 and Fibrocell's other product candidates; the potential benefits of the clinical trial research grant from the OOPD; the potential benefits of Fast Track Designation, Orphan Drug Designation and Rare Pediatric Disease Designation; and other statements regarding Fibrocell's future operations, financial performance and financial position, prospects, strategies, objectives and other future events.

Forward-looking statements are based upon management's current expectations and assumptions and are subject to a number of risks, uncertainties and other factors that could cause actual results and events to differ materially and adversely from those indicated herein including, among others:

uncertainties and delays relating to the initiation, enrollment and completion of clinical trials; whether clinical trial results will validate and support the safety and efficacy of Fibrocell's product candidates; unanticipated or excess costs relating to the development of Fibrocell's gene therapy product candidates; Fibrocell's ability to obtain additional capital to continue to fund operations; uncertainties associated with being able to identify, evaluate and complete any strategic transaction or alternative; the impact of the announcement of the Board of Directors' review of strategic alternatives, as well as any strategic transaction or alternative that may be pursued, on the Company's business, including its financial and operating results and its employees; Fibrocell's ability to maintain its collaboration with Precigen, Inc.; and the risks, uncertainties and other factors discussed under the caption "Item 1A. Risk Factors" in Fibrocell's most recent Form 10-K filing and Form 10-Q filings. As a result, you are cautioned not to place undue reliance on any forward-looking statements. While Fibrocell may update certain forward-looking statements from time to time, Fibrocell specifically disclaims any obligation to do so, whether as a result of new information, future developments or otherwise.

Investor & Media Relations Contact:
Karen Casey
484.713.6133
kcasey@fibrocell.com

FIBROCELL

Fibrocell Science Inc.