For Immediate Release

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FDA APPROVES NEW LIQUID FORM OF TREATMENT FOR PATIENTS WITH ACUTE LYMPHOBLASTIC LEUKEMIA (ALL) AS PART OF A COMBINATION REGIMEN

PURIXAN™ (mercaptopurine) Oral Suspension Finally Makes Accurate Dosing Simple for Healthcare Professionals to Achieve—Especially with Children

<Franklin, TN; April 30, 2014> Rare Disease Therapeutics, Inc. (RDT), the producer of orphan pharmaceuticals for the treatment of rare diseases, announced today that they have received FDA approval for PURIXAN™ (mercaptopurine) 20 mg/mL oral suspension---a new, easier-to-dose, liquid form of an established treatment for patients with Acute Lymphoblastic Leukemia (ALL). Unlike the 50 mg tablet form that healthcare professionals often split to provide children with the desired dose, the raspberry-flavored PURIXAN liquid enables the measurement of precise doses that can easily be administered to pediatric and adult patients.

On April 28, 2014, in a written announcement to Clinical Oncologists, the US Food and Drug administration pointed out that "Compared to tablets, a suspension offers the advantage of more accurately delivering the desired dose to children with a wide range of weights using a consistent administration schedule. A suspension will allow more flexibility in adjusting the dose" and that "a commercially produced suspension is more likely to provide a more consistent dose of 6-mercaptopurine than *ad hoc* compounded formulations.[1]"

"Our management shares FDA's opinion that a 50 mg tablet formulation is not ideal for treating the age and weight range of patients with ALL—especially children under 6 years of age," responded Milton Ellis, President & CEO of RDT. "That's why our team has been so enthusiastic about working with FDA to bring PURIXAN oral suspension to market; and why we are also grateful for the close collaboration of Nova Laboratories, Ltd., the inventor and manufacturer of the sophisticated formulation that gives PURIXAN its special clinical advantages."

Approximately 6,020 people in the United States are diagnosed with new cases of ALL each year, and the risk is highest in children under 5 years of age according to the American Cancer Society[2].

"ALL therapy includes 6-mercaptopurine taken orally every day for 2-3 years. Until now, the only FDA approved formulation of this drug available in the US has been pills, which can be very hard for young children to take. This oral-suspension

formulation should be much easier for young children to take and help parents to make sure that their children get the treatment that they need to cure ALL" commented Stephen Hunger, MD, Chairman of the Children's Oncology Group ALL Disease Committee, Professor and Ergen Family Chair in Pediatric Cancer, and Director, Center for Cancer and Blood Disorders at the University of Colorado School of Medicine.

Because PURIXAN is designated an Orphan Drug, RDT has been granted a period of exclusivity in marketing this innovation and will be distributing PURIXAN oral suspension through the firm AnovoRx Distribution, LLC in Memphis, Tennessee--which can be contacted at 1-888-470-0904.

The most common adverse reaction (> 20% of patients) is myelosupression including anemia, neutropenia, and thrombocytopenia. Less common (5-20% of patients) adverse reactions include elevated transaminases, elevated bilirubin, intestinal ulceration, nausea, vomiting, anorexia, diarrhea and rashes.

See package insert for important safety information and full prescribing information about PURIXAN.
http://www.accessdata.fda.gov/drugsatfda_docs/label/2014/205919s000lbl.pdf

Forward-Looking Statement
This press release includes "forward-looking statements" within the meaning of the safe harbor provisions of the United States Private Securities Litigation Reform Act of 1995. These statements are based upon the current beliefs and expectations of RDT management and are subject to significant risks and uncertainties. There can be no guarantees with respect to pipeline products that the products will receive the necessary regulatory approvals or that they will prove to be commercially successful. If underlying assumptions prove inaccurate or risks or uncertainties materialize, actual results may differ materially from those set forth in the forward-looking statements.

Risks and uncertainties include but are not limited to, general industry conditions and competition; general economic factors, including interest rate and currency exchange rate fluctuations; the impact of pharmaceutical industry regulation and health care legislation in the United States and internationally; global trends toward health care cost containment; technological advances, new products and patents attained by competitors; challenges inherent in new product development, including obtaining regulatory approval; RDT's ability to accurately predict future market conditions; manufacturing difficulties or delays; financial instability of international economies and sovereign risk; dependence on the effectiveness of Nova Laboratories, Ltd. patents and other protections for innovative products; and the exposure to litigation, including patent litigation, and/or regulatory actions.

RDT undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise.

About Rare Disease Therapeutics, Inc.

Rare Disease Therapeutics, Inc., specializes in filling the very specific and special medical needs of a small population of adults and children seeking life-preserving therapies for rare diseases. Many of these diseases are not immediately recognizable to even the most astute physicians; therefore, the role of Rare Disease Therapeutics, Inc., must include creating awareness of such diseases and their diagnoses.

Rare Disease Therapeutics, Inc., is a company built on the hope and premise of providing options where there are no options, and providing therapies where there are no therapies, through creative strategic alliances, as well as relationships with the government, research institutions, physicians, scientists, and manufacturers.

Rare Disease Therapeutics, Inc., was originally founded in 1991 as Orphan Pharmaceuticals, U.S.A., Inc., and is strategically located in Nashville, Tennessee.

Please visit us at www.raretx.com

References:

1. FDA release dated 04/29/14
2. ACS web site:
http://www.cancer.org/cancer/leukemia-acutelymphocyticallinadults/detailedguide/leukemia-acute-lymphocytic-key-statistics

Source: Rare Disease Therapeutics, Inc.