RedHill Biopharma Announces FDA Orphan Drug Designation for RHB-204 for the Treatment of NTM Infections

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RHB-204 Phase 3 study in pulmonary NTM infections is planned to be initiated in the coming weeks

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Orphan Drug Designation, along with RHB-204's FDA QIDP priority designation, will extend U.S. potential market exclusivity to a total of 12 years

TEL AVIV, Israel and RALEIGH, N.C., Oct. 14, 2020 (GLOBE NEWSWIRE) -- RedHill Biopharma Ltd. (Nasdaq: RDHL) ("RedHill" or the "Company"), a specialty biopharmaceutical company, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to RHB-204 for the treatment of nontuberculous mycobacteria (NTM) disease.

"NTM infections are resistant to most antibiotics and are extremely challenging to treat. With no FDA-approved first-line therapy available to patients today, there is a tremendous urgent need for new treatments for this debilitating and potentially fatal infectious disease," **said Patricia Anderson, RedHill's VP Regulatory Affairs**. "Along with the QIDP designation already granted to RHB-204 by the FDA, the Orphan Drug Designation extends potential market exclusivity to a total of 12 years."

A Phase 3 study to evaluate the safety and efficacy of RHB-204 in patients with pulmonary NTM infections caused by *Mycobacterium avium* Complex (MAC) is planned to be initiated in the coming weeks in the U.S.

The multi-center, randomized, double-blind, placebo-controlled, parallel-group Phase 3 study, under ongoing discussion with the FDA, is planned to be conducted at up to 40 sites across the U.S. and aims to enroll 125 patients, randomized at a 3:2 ratio to receive either RHB-204 or placebo. The study is planned to evaluate the safety and efficacy of RHB-204, evaluating patient-reported outcomes and sputum culture conversion (SCC) by Month 6 of treatment with RHB-204, compared to placebo, and patients will continue to receive treatment for 12 months from SCC.

The FDA grants orphan status to drugs intended to treat rare disorders that affect fewer than 200,000 people in the U.S. The designation provides various development incentives to the drug developer, including extended market exclusivity upon FDA approval, prescription drug user fee (PDUFA) waivers and tax credits for qualified clinical testing.

RHB-204 was previously granted a Qualified Infectious Disease Product (QIDP) designation by the FDA, providing for eligibility for Fast-Track development, NDA priority review and a five-year extension of U.S. market exclusivity, if approved. The Orphan Drug Designation extends U.S. market exclusivity for RHB-204 by an additional seven years, for a potential total of 12 years upon FDA approval.

About Pulmonary Nontuberculous Mycobacteria (NTM) Infections

Pulmonary nontuberculous mycobacteria (NTM) disease is a chronic and debilitating lung disease caused by ubiquitous environmental bacteria, found in soil as well as natural and engineered water systems. The most common NTM symptoms include fever, weight loss, chest pain, and blood in sputum[1]. NTM infections can lead to recurring cases of bronchitis and pneumonia and can, in some cases, lead to respiratory failure[2]. Although rare, the incidence and prevalence of pulmonary NTM disease are increasing in many areas of the world[3]. There were an estimated 110,000 pulmonary NTM disease patients in the U.S. in 2017[4]. Pulmonary manifestations account for 80-90% of all NTM-associated diseases[5], and approximately 80% of pulmonary NTM infections are caused by Mycobacterium avium Complex (MAC)[6].

Treatment of NTM infection can be difficult, with no FDA-approved first-line standard-of-care therapy. It requires multiple antibiotics and an extended treatment course due to the risk of development of resistance[7]. Many patients fail these types of therapies and more than half will have either recurring disease or a new infection after completing treatment[8]. Thus, new treatment options for NTM are urgently needed.

About RHB-204

RHB-204 is a proprietary, fixed-dose oral capsule containing a combination of clarithromycin, rifabutin, and clofazimine, developed for the treatment of pulmonary NTM infections caused by *Mycobacterium avium* Complex (MAC). RHB-204 was granted both FDA Orphan Drug Designation for the treatment of NTM disease and QIDP Designation under the Generating Antibiotic Incentives Now Act (GAIN Act), extending U.S. market exclusivity for RHB-204 to a potential total of 12 years to be granted at the time of FDA approval. RHB-204 is also covered by U.S. patents which extend patent protection until 2029 and a pending U.S. patent application which, if allowed, could extend RHB-204 patent protection until 2041.

About RedHill Biopharma

RedHill Biopharma Ltd. (Nasdaq: RDHL) is a specialty biopharmaceutical company primarily focused on gastrointestinal and infectious diseases. RedHill promotes the gastrointestinal drugs, **Movantik®** for opioid-induced constipation in adults[9], **Talicia®** for the treatment of *Helicobacter pylori (H. pylori)* infection in adults[10], and **Aemcolo®** for the treatment of travelers' diarrhea in adults [11]. RedHill's key clinical late-stage development programs include: (i) **RHB-204**, with a planned Phase 3 study for pulmonary nontuberculous mycobacteria (NTM) infections; (ii) **opaganib (Yeliva®)**, a first-in-class SK2 selective inhibitor targeting multiple indications with a Phase 2/3 program for COVID-19 and Phase 2 studies for prostate cancer and cholangiocarcinoma ongoing; (iii) **RHB-104**, with positive results from a first Phase 3 study for Crohn's disease; (iv) **RHB-102** (**Bekinda®**), with positive results from a Phase 3 study for acute gastroenteritis and gastritis and positive results from a Phase 2 study for IBS-D; (v) **RHB-107**, a Phase 2-stage first-in-class, serine protease inhibitor, targeting cancer and inflammatory gastrointestinal diseases and is also being evaluated for COVID-19 and (vi) **RHB-106**, an encapsulated bowel preparation. More information about the Company is available at www.redhillbio.com.

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such statements may be preceded by the words "intends," "may," "will," "plans," "expects," "anticipates," "projects," "predicts," "estimates," "aims," "believes," "hopes," "potential" or similar words. Forward-looking statements are based on certain assumptions and are subject to various known and unknown risks and uncertainties, many of which are beyond the Company's control and cannot be predicted or quantified, and consequently, actual results may differ materially from those expressed or implied by such forward-looking statements. Such risks and uncertainties include, without limitation, the risk that the Company will not initiate the Phase 3 clinical study in all or part of the sites in the U.S. or will be delayed; the risk that the U.S. Phase 3 clinical study evaluating RHB-204 will not be successful or, if successful, will not suffice for regulatory marketing approval without the need for additional clinical and/or other studies; as well as risks and uncertainties associated with (i) the initiation, timing, progress and results of the Company's research, manufacturing, pre-clinical studies, clinical trials, and other therapeutic candidate development efforts, and the timing of the commercial launch of its commercial products and ones it may acquire or develop in the future; (ii) the Company's ability to advance its therapeutic candidates into clinical trials or to successfully complete its pre-clinical studies or clinical trials or the development of a commercial companion diagnostic for the detection of MAP; (iii) the extent and number and type of additional studies that the Company may be required to conduct and the Company's receipt of regulatory approvals for its therapeutic candidates, and the timing of other regulatory filings, approvals and feedback; (iv) the manufacturing, clinical development, commercialization, and market acceptance of the Company's therapeutic candidates and Talicia®; (v) the Company's ability to successfully commercialize and promote Talicia®, and Aemcolo® and Movantik®*; (vi) the Company's ability to est ablish and maintain corporate collaborations; (vii) the Company's ability to acquire products approved for marketing in the U.S. that achieve commercial success and build its own marketing and commercialization capabilities; (viii) the interpretation of the properties and characteristics of the Company's therapeutic candidates and the results obtained with its therapeutic candidates in research, pre-clinical studies or clinical trials; (ix) the implementation of the Company's business model, strategic plans for its business and therapeutic candidates; (x) the scope of protection the Company is able to establish and maintain for intellectual property rights covering its therapeutic candidates and its ability to operate its business without infringing the intellectual property rights of others; (xi) parties from whom the Company licenses its intellectual property defaulting in their obligations to the Company; (xii) estimates of the Company's expenses, future revenues , capital requirements and needs for additional financing; (xiii) the effect of patients suffering adverse experiences using investigative drugs under the Company's Expanded Access Program; (xiv) competition from other companies and technologies within the Company's industry ; and (xv) the hiring and employment commencement date of executive managers. More detailed information about the Company and the risk factors that may affect the realization of forward-looking statements is set forth in the Company's filings with the Securities and Exchange Commission (SEC), including the Company's Annual Report on Form 20-F filed with the SEC on March 4, 2020. All forward-looking statements included in this press release are made only as of the date of this press release. The Company assumes no obligation to update any written or oral forward-looking statement, whether as a result of new information, future events or otherwise unless required by law.*

Company contact:
Adi Frish
Senior VP Business Development & Licensing
RedHill Biopharma
+972-54-6543-112
adi@redhillbio.com

IR contact (U.S.):
Timothy McCarthy, CFA, MBA
Managing Director, Relationship Manager
LifeSci Advisors, LLC
+1-212-915-2564
tim@lifesciadvisors.com

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[9] Full prescribing information for Movantik® (naloxegol) is available at: www.Movantik.com.

[10] Full prescribing information for Talicia® (omeprazole magnesium, amoxicillin and rifabutin) is available at: www.Talicia.com.

[11] Full prescribing information for Aemcolo® (rifamycin) is available at: www.Aemcolo.com.

Source: RedHill Biopharma Ltd.