



January 11, 2017

## **RedHill Biopharma Announces QIDP Fast-Track Designation Granted by FDA to RHB-104 for Nontuberculous Mycobacteria Infections**

- | **RHB-104 has been granted Qualified Infectious Disease Product (QIDP) designation by the U.S. FDA for the treatment of Nontuberculous Mycobacteria (NTM) Infection**
  
- | **Under FDA's Generating Antibiotic Incentives Now (GAIN) Act, QIDP designation allows for Fast-Track status and Priority Review, potentially leading to a shorter NDA review time by the FDA, and, if approved, an additional five years of U.S. market exclusivity on top of the standard exclusivity period**
  
- | **NTM infections can occur throughout the body, although pulmonary infections, lymphadenitis, and skin and soft tissue infections are the most common; NTM infections have been increasing worldwide over the past two decades; Treatment is typically prolonged and requires multi-drug regimens due to the risk of development of resistance**
  
- | **A first Phase III clinical study with RHB-104 for the treatment of Crohn's disease (the MAP US study) is ongoing in the U.S. and additional countries; Increasing evidence supports the hypothesis that Crohn's disease, and potentially other autoimmune diseases, are related to *Mycobacterium avium subspecies paratuberculosis* (MAP) infection in susceptible patients**

TEL-AVIV, Israel, Jan. 11, 2017 (GLOBE NEWSWIRE) -- RedHill Biopharma Ltd. (NASDAQ:RDHL) (TASE:RDHL) ("RedHill" or the "Company"), a specialty biopharmaceutical company primarily focused on the development and commercialization of late clinical-stage, proprietary, orally-administered, small molecule drugs for gastrointestinal and inflammatory diseases and cancer, today announced that RHB-104 has been granted Qualified Infectious Disease Product (QIDP) designation by the U.S. FDA for the treatment of Nontuberculous Mycobacteria (NTM) infections. The QIDP designation was granted under the FDA's Generating Antibiotic Incentives Now (GAIN) Act, which is intended to encourage development of new antibiotic drugs for the treatment of serious or life-threatening infections.

The granted QIDP designation allows RedHill to benefit from Fast-Track status, with an expedited development pathway for RHB-104 for the treatment of NTM infections, as well as Priority Review, which provides for a shorter review time by the FDA of a future potential marketing application. If approved for the treatment of NTM infections, RHB-104 would also receive an additional five years of U.S. market exclusivity on top of the standard exclusivity period.

RHB-104 is a proprietary and potentially groundbreaking antibiotic combination therapy in oral capsule formulation, with potent intracellular, anti-mycobacterial and anti-inflammatory properties.

In light of the QIDP designation, RedHill will consult with the FDA regarding its RHB-104 development program for NTM infections.

NTM infections can occur throughout the body, although pulmonary infections, lymphadenitis, and skin and soft tissue infections are the most commonly affected areas. There are a number of risk factors that can increase a person's chances of acquiring NTM infections, including history of bronchiectasis, chronic obstructive pulmonary disease, smoking, HIV/AIDS, use of immunosuppressive drugs and certain genetic conditions, such as cystic fibrosis and alpha-1 antitrypsin deficiency.

Nontuberculous mycobacteria are ubiquitous organisms commonly isolated from the environment and identified in drinking water and soil. Patients suffering from NTM infection are at increased risk for reinfection<sup>1</sup>. NTM infections have been increasing worldwide over the past two decades<sup>2</sup>, while treatment of NTM infection remains difficult and entails multiple antibiotics and an extended treatment course due to the risk of development of resistance.

A first Phase III study with RHB-104 for the treatment of Crohn's disease (the MAP US study) is currently ongoing in the U.S. and additional countries. The development of RHB-104 is based on increasing evidence supporting the hypothesis that Crohn's disease, and potentially other autoimmune diseases, are related to *Mycobacterium avium subspecies*

*paratuberculosis* (MAP) infection in susceptible patients.

Recently, RedHill also completed a Phase IIa, proof-of-concept clinical study, evaluating RHB-104 as an add-on therapy to interferon beta-1a in patients treated for relapsing remitting multiple sclerosis (the CEASE MS study). Top-line final results from the CEASE MS study suggest meaningful positive safety and clinical signals upon 24 weeks of treatment with RHB-104 as an add-on therapy, thereby supporting further clinical development.

#### **About RHB-104:**

Currently in a first Phase III study for the treatment of Crohn's disease (the MAP US study), RHB-104 is a proprietary and potentially groundbreaking oral antibiotic combination therapy, with potent intracellular, anti-mycobacterial and anti-inflammatory properties. RHB-104 is based on increasing evidence supporting the hypothesis that Crohn's disease is caused by *Mycobacterium avium subspecies paratuberculosis* (MAP) infection in susceptible patients. Clinical trials conducted with earlier formulations of RHB-104 include an Australian Phase III study conducted by Pharmacia/Pfizer. RedHill has conducted several supportive studies with the current formulation of RHB-104 and a long-term population pharmacokinetic (pop-PK) study is ongoing as part of the Phase III MAP US study. RHB-104 is covered by several issued and pending patents. RedHill has also completed a Phase IIa, proof-of-concept clinical study, evaluating RHB-104 as an add-on therapy to interferon beta-1a in patients treated for relapsing remitting multiple sclerosis (the CEASE MS study). Top-line final results from the CEASE MS study suggest meaningful positive safety and clinical signals upon 24 weeks of treatment with RHB-104 as an add-on therapy, thereby supporting further clinical development. RHB-104 was also granted QIDP designation for the treatment of Nontuberculous Mycobacteria (NTM) infections, allowing for Fast-Track status and Priority Review, potentially leading to a shorter NDA review time by FDA, and, if approved, an additional five years of U.S. market exclusivity.

#### **About RedHill Biopharma Ltd.:**

RedHill Biopharma Ltd. (NASDAQ:RDHL) (TASE:RDHL) is a specialty biopharmaceutical company headquartered in Israel, primarily focused on the development and commercialization of late clinical-stage, proprietary, orally-administered, small molecule drugs for the treatment of gastrointestinal and inflammatory diseases and cancer. RedHill has a U.S. co-promotion agreement with Concordia for **Donnatal**<sup>®</sup>, a prescription oral adjunctive drug used in the treatment of IBS and acute enterocolitis. RedHill's clinical-stage pipeline includes: (i) **RHB-105** - an oral combination therapy for the treatment of *Helicobacter pylori* infection with successful results from a first Phase III study; (ii) **RHB-104** - an oral combination therapy for the treatment of Crohn's disease with an ongoing first Phase III study and a completed proof-of-concept Phase IIa study for multiple sclerosis; (iii) **BEKINDA**<sup>®</sup> (**RHB-102**) - a once-daily oral pill formulation of ondansetron with an ongoing Phase III study for acute gastroenteritis and gastritis and an ongoing Phase II study for IBS-D; (iv) **RHB-106** - an encapsulated bowel preparation licensed to Salix Pharmaceuticals, Ltd.; (v) **YELIVA**<sup>®</sup> (**ABC294640**) - a Phase II-stage, orally-administered, first-in-class SK2 selective inhibitor targeting multiple oncology, inflammatory and gastrointestinal indications; (vi) **MESUPRON** - a Phase II-stage first-in-class, orally-administered uPA inhibitor, targeting gastrointestinal and other solid tumors and (vii) **RIZAPORT**<sup>®</sup> (**RHB-103**) - an oral thin film formulation of rizatriptan for acute migraines, with a U.S. NDA currently under discussion with the FDA and marketing authorization received in Germany in October 2015. More information about the Company is available at: [www.redhillbio.com](http://www.redhillbio.com).

<sup>1</sup> Falkinham JO, *Environmental sources of nontuberculous mycobacteria*, Clin. Chest Med. 2015, 36, 35-41.

<sup>2</sup> Adjemian J et al., *Prevalence of Nontuberculous Mycobacterial Lung Disease in U.S. Medicare Beneficiaries*, Am J Respir Crit Care Med. 2012 Apr 15; 185(8): 881-886.; Brown-Elliott et al. *Antimicrobial Susceptibility Testing, Drug Resistance Mechanisms, and Therapy of Infections with Nontuberculous Mycobacteria*, [Clin Microbiol Rev](http://www.clinmicrobiolrev.com). 2012 Jul; 25(3): 545-582

*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, risks and uncertainties associated with (i) the initiation, timing, progress and results of the Company's research, manufacturing, preclinical studies, clinical trials, and other therapeutic candidate development efforts; (ii) the Company's ability to advance its therapeutic candidates into clinical trials or to successfully complete its preclinical studies or clinical trials; (iii) the extent and number 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; (v) the Company's ability to establish and maintain corporate collaborations; (vi) 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; (vii) the interpretation of the properties and characteristics of the Company's therapeutic candidates and of the results obtained with its therapeutic candidates in research, preclinical studies or clinical trials; (viii) the implementation of*

*the Company's business model, strategic plans for its business and therapeutic candidates; (ix) 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; (x) parties from whom the Company licenses its intellectual property defaulting in their obligations to the Company; (xi) estimates of the Company's expenses, future revenues capital requirements and the Company's needs for additional financing; (xii) competitive companies and technologies within the Company's industry; and (xiii) the impact of the political and security situation in Israel on the Company's business. 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 February 25, 2016. All forward-looking statements included in this Press Release are made only as of the date of this Press Release. We assume no obligation to update any written or oral forward-looking statement unless required by law.*

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