RedHill Biopharma Announces Last Patient Assessed for Primary Endpoint in RHB-104 Phase III Study for Crohn's Disease

May 7, 2018

- **Top-line results from the Phase III study with RHB-104 for Crohn's disease are expected to be announced in approximately three months**
- **Worldwide sales of Crohn's disease therapies are estimated to exceed $10 billion in 2018**
- **Top-line results from the confirmatory Phase III study with TALICIA® for *H. pylori* infection are expected in Q4/2018**
- **RedHill will host a webcast for the investment community on RHB-104 for Crohn's disease on Tuesday, May 15, 2018, at 8:00 am EDT**

TEL-AVIV, Israel and RALEIGH, N.C., May 07, 2018 (GLOBE NEWSWIRE) -- RedHill Biopharma Ltd. (NASDAQ:RDHL) (Tel-Aviv Stock Exchange:RDHL) ("RedHill" or the "Company"), a specialty biopharmaceutical company primarily focused on late clinical-stage development and commercialization of proprietary drugs for gastrointestinal diseases, today announced that the last patient enrolled in the first Phase III study with RHB-104 for Crohn's disease (the MAP US study) has completed 26 weeks of treatment for primary endpoint evaluation. Top-line results are expected to be announced in approximately three months.

The MAP US study is a randomized, double-blind, placebo-controlled first Phase III study evaluating the safety and efficacy of RHB-104 in subjects with moderately to severely active Crohn's disease (defined as Crohn's Disease Active Index (CDAI) between 220 and 450). The primary endpoint is disease remission, defined as CDAI value of less than 150 at week 26. The study has enrolled 331 patients across clinical sites in the U.S., Canada, Europe, Israel, Australia and New Zealand.

A review of the blended efficacy rate of the current blinded data suggests that the total number of treatment successes at this point in the study is consistent with the predefined expected treatment outcome and protocol-defined 15% treatment effect (RHB-104 36% vs. placebo 21%). The blended remission rate of the currently blinded data has been consistently within or superior to our pre-specified protocol defined assumptions, indicating potential study success assuming the placebo and RHB-104 remission rates in the study are in line with trial assumptions. Placebo remission rates in similar, but not identical, pivotal studies in Crohn's disease range from approximately 7% to approximately 25% [1] with the two most recently approved therapies at 7% (Entyvio® (vedolizumab))[2] and 20% (Stelara® (ustekinumab))[3]. The Company remains blinded to the data and has no visibility into the actual treatment effect.

RHB-104 is a potentially ground-breaking, proprietary, orally-administered antibiotic combination therapy, with potent intracellular, antimycobacterial and anti-inflammatory properties. RHB-104 is based on the hypothesis that Crohn's disease is caused by *Mycobacterium avium subspecies paratuberculosis* (MAP) infection in susceptible patients.

An independent Data and Safety Monitoring Board (DSMB) held two pre-planned meetings and unanimously recommended to continue the study, with no safety concerns. The first DSMB meeting reviewed safety data from the study and the second DSMB meeting reviewed safety and efficacy data from the first 222 subjects who had completed week 26 assessments of the study.

In addition, an open-label extension Phase III study (MAP US2 study) is ongoing to evaluate the safety and efficacy of RHB-104 in subjects who remain with active Crohn's disease (CDAI ≥ 150) after 26 weeks of blinded study therapy in the ongoing Phase III MAP US study.

Additional clinical studies are likely to be required to support a U.S. New Drug Application (NDA) for RHB-104, if filed. If the MAP US study results are positive, RedHill will meet with key opinion leaders and the U.S. Food and Drug Administration (FDA) to present the data package and discuss the preferred path to potential approval.

Approximately 1.5 million people were diagnosed with Crohn's disease worldwide in 2017[4]. Worldwide sales of Crohn's disease therapies are estimated to exceed $10 billion in 2018[5].

RedHill will host an Analyst and Investor Webcast on RHB-104 for Crohn's disease on Tuesday, May 15, 2018, at 8:00 a.m. EDT.

Members of RedHill's executive team will be joined by key opinion leaders who will discuss RHB-104, the MAP US study, Crohn's disease, the current treatment landscape and potential market. A question and answer session will be held following the presentations.

The conference call, including a slide presentation, will be broadcasted live and available for replay on the Company's website, http://ir.redhillbio.com/events, for 30 days. Please access the website at least 15 minutes ahead of the conference call to register, download, and install any necessary audio software.

Participants who wish to ask questions during the event can do so by telephone. To participate in the conference call, please dial one of the following numbers 5-10 minutes prior to the start of the call: **United States: +1-800-263-0877; International: +1-646-828-8143; and Israel: +972-3-376-1315. The access code for the call is 1951893.**

The clinical studies with RHB-104 are registered on www.ClinicalTrials.gov, a web-based service of the U.S. National Institute of Health, which provides access to information on publicly and privately-supported clinical studies.

About RHB-104:
Currently in a first Phase III study for the treatment of Crohn's disease (MAP US study), with top-line results expected in mid-2018, RHB-104 is a potentially ground-breaking, proprietary, orally-administered antibiotic combination therapy, with potent intracellular, antimycobacterial and anti-inflammatory properties. RHB-104 is based on the hypothesis that Crohn's disease is caused by *Mycobacterium avium subspecies paratuberculosis* (MAP) infection in susceptible patients. The development of RHB-104 is consistent with the growing awareness of the possibility that a bacterially-induced dysregulated immune system may contribute to the pathogenesis of various autoimmune diseases of unknown etiology. 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. Additionally, an open-label extension Phase III study (MAP US2 study) is ongoing to assess the safety and efficacy of RHB-104 in subjects who have completed week 26 assessments in the ongoing Phase III MAP US study and remain with active Crohn's disease (CDAI ≥ 150). RHB-104 is covered by several issued and pending patents. RHB-104 was granted Qualified Infectious Disease Product (QIDP) designation by the U.S. FDA for the treatment of nontuberculous mycobacteria (NTM) infections, providing a Fast-Track development pathway, as well as NDA Priority Review, and an additional five years of U.S. market exclusivity, if approved. A pivotal Phase III study with RHB-104 for NTM infections is planned to be initiated. RedHill also completed a Phase IIa, proof-of-concept clinical study evaluating RHB-104 as an add-on therapy to interferon beta-1a in subjects treated for relapsing-remitting multiple sclerosis (CEASE MS study), supporting additional studies.

About RedHill Biopharma Ltd.:
RedHill Biopharma Ltd. (NASDAQ:RDHL) (Tel-Aviv Stock Exchange:RDHL) is a specialty biopharmaceutical company, primarily focused on the development and commercialization of late clinical-stage, proprietary drugs for the treatment of gastrointestinal diseases. RedHill commercializes and promotes three gastrointestinal products in the U.S.: **Donnatal®-** a prescription oral adjunctive drug used in the treatment of IBS and acute enterocolitis; **Esomeprazole Strontium Delayed-Release Capsules 49.3 mg -** a prescription proton pump inhibitor indicated for adults for the treatment of gastroesophageal reflux disease (GERD) and other gastrointestinal conditions; and **EnteraGam®-** a medical food intended for the dietary management, under medical supervision, of chronic diarrhea and loose stools. RedHill's key clinical-stage development programs include: (i) **TALICIA® (RHB-105)** for the treatment of *Helicobacter pylori* infection with an ongoing confirmatory Phase III study and positive results from a first Phase III study; (ii) **RHB-104** with an ongoing first Phase III study for Crohn's disease and a planned pivotal Phase III study for nontuberculous mycobacteria (NTM) infections; (iii) **BEKINDA® (RHB-102)** with positive results from a Phase III study for acute gastroenteritis and gastritis and positive results from a Phase II study for IBS-D; (iv) **YELIVA® (ABC294640),** a first-in-class SK2 selective inhibitor, targeting multiple oncology, inflammatory and gastrointestinal indications, with an ongoing Phase IIa study for cholangiocarcinoma; (v) **RHB-106,** an encapsulated bowel preparation licensed to Salix Pharmaceuticals, Ltd. and (vi) **RHB-107 (formerly MESUPRON),** a Phase II-stage first-in-class, serine protease inhibitor, targeting cancer and inflammatory gastrointestinal diseases. 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, 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 successfully promote Donnatal® and Esomeprazole Strontium Delayed-Release Capsules 49.3 mgand commercialize EnteraGam®; (vi) the Company's ability to establish 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, preclinical 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; and (xiv) competition from other companies and technologies within the Company's industry. 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 SECon February 22, 2018. 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.

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[1] Remicade® ((infliximab) package insert
https://www.accessdata.fda.gov/drugsatfda_docs/label/2013/103772s5359lbl.pdf.

[2] Entyvio® (vedolizumab) package insert.
https://www.accessdata.fda.gov/drugsatfda_docs/label/2014/125476s000lbl.pdf.

[3] Stelara® (ustekinumab) package insert.
https://www.accessdata.fda.gov/drugsatfda_docs/label/2016/761044lbl.pdf.

[4] GlobalData PharmaPoint: Crohn's Disease - Global Forecast 2016–2026, September 2017.
[5] GlobalData PharmaPoint: Crohn's Disease - Global Forecast 2016–2026, September 2017.

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