NEWS RELEASE

RedHill's Opaganib Receives FDA Rare Pediatric Disease Designation for Neuroblastoma in Addition to Current Orphan Drug Designation

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The U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation to opaganib[1] for the treatment of neuroblastoma, a type of cancer most commonly affecting babies and young children

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Rare pediatric disease designation provides for a Priority Review Voucher (PRV) subject to certain conditions

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This new designation is in addition to opaganib's current neuroblastoma orphan drug designation, providing for potential benefits such as accelerated development and review times, FDA Prescription Drug User Fee Act (PDUFA) application fee waivers, tax credits and seven-years' marketing exclusivity, if approved

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New preclinical data, presented at the 2026 American Association for Cancer Research (AACR) Annual Meeting, showed positive effects of opaganib as a potential add-on therapy in models of neuroblastoma and triple-negative breast cancer (TNBC)[2]

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The neuroblastoma market is expected to be valued at approximately $3.5 billion in 2032[3]

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Opaganib is a novel, potentially broad acting, oral, small molecule drug with demonstrated safety & efficacy profiles[4]. It is in development for multiple oncology, viral (including Ebola virus disease (EVD)), inflammatory and diabetes and obesity-related indications

RALEIGH, N.C. and TEL-AVIV, Israel, June 9, 2026 /PRNewswire/ -- 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 rare pediatric disease (RPD) designation to opaganib for treatment of neuroblastoma (NB).

"Receiving a cancer diagnosis is always distressing, but when it involves your child, it becomes profoundly

devastating. There is an ongoing necessity to explore new alternatives that can augment treatment and enhance results for neuroblastoma, the most prevalent cancer in infants. In data from models of high-risk NB (HRNB), presented at AACR 2026, we saw positive effects of opaganib as a potential add-on to chemotherapy, showing an ability to directly destabilize a key oncogenic driver of neuroblastoma and other solid tumors, n-Myc, through increased ceremide production enhancing programmed cancer cell death (apoptosis)," **said Dr. Mark Levitt, Chief Scientific Officer at RedHill**. "This rare pediatric disease designation, supported by data from NB and other preclinical oncology models, along with a clinically demonstrated safety and tolerability profile adds to our belief that opaganib holds promise for improving outcomes in treating pediatric NB. We aim to further advance development following ongoing discussions with Penn State University and the Beat Childhood Cancer consortium."

The FDA grant of rare pediatric disease designation to opaganib provides for a Priority Review Voucher (PRV), subject to certain conditions, and with opaganib's current neuroblastoma orphan drug designation also allows for the potential for seven years' marketing exclusivity, if approved, accelerated development and review times, FDA Prescription Drug User Fee Act (PDUFA) application fee waivers and tax credits. The neuroblastoma market is expected to be valued at approximately $3.5 billion in 2032.

Opaganib is a novel, potentially broad acting, oral, small molecule sphingosine kinase-2 (SPHK2) selective inhibitor drug with demonstrated safety & efficacy profiles. It is in development for multiple oncology, viral (including Ebola virus disease (EVD), inflammatory and diabetes and obesity-related indications.

About Neuroblastoma

Neuroblastoma is a type of cancer most commonly affecting babies and young children. While rare, neuroblastoma is the most common infancy cancer with ~5,500 global pediatric cases per year in children aged 0–14. It accounts for 10% of childhood cancers and 15% of pediatric cancer-related deaths in the U.S.[5,6]. Around 750 children in the United States are diagnosed with neuroblastoma each year[7]. Approximately half of all neuroblastoma patients have high risk (HRNB) disease which has an overall five-year survival of ~50%[8].

Neuroblastoma originates from immature nerve cells (neuroblasts), and most often forms in the adrenal glands - small organs that sit on top of the kidneys - but can also start in nerve cells in the abdomen, chest, neck, or pelvis. The exact cause of neuroblastoma is not well understood, but genetic mutations and abnormalities are known to play a role. Some cases may be linked to genetic syndromes or family history, although most occur sporadically without a clear inherited pattern.

About Opaganib (ABC294640)

Opaganib is a proprietary first-in-class investigational, orally administered sphingosine kinase-2 (SPHK2) selective inhibitor drug. Potentially broad-acting, it is in development for multiple oncology, viral (including Ebola virus disease), inflammatory, metabolic (diabetes and obesity) and additional indications.

Opaganib's suggested mechanism of action, **published** in the journal Drug Design, Development and Therapy, is host-directed and potentially broad-acting and is expected to maintain its effect against emerging viral variants. Opaganib is thought to work through the inhibition of multiple pathways, the induction of autophagy and apoptosis, and disruption of viral replication, through simultaneous inhibition of three sphingolipid-metabolizing enzymes in human cells (SPHK2, DES1 and GCS).

Opaganib has received Orphan Drug designation from the FDA for the treatment of neuroblastoma and cholangiocarcinoma. A Bayer-supported 80-patient placebo-controlled randomized Phase 2 study is ongoing to evaluate the efficacy of opaganib in combination with Bayer's darolutamide in men with metastatic castrate-resistant prostate cancer (mCRPC), testing the potentially enhancing effect of opaganib in patients with a poor prognosis[9]. Opaganib also has a Phase 1 chemoradiotherapy study protocol ready for FDA-IND submission.

Opaganib has demonstrated its safety and tolerability profile in more than 470 participants in multiple clinical

studies and expanded access use, including a large global Phase 2/3 study in hospitalized patients with moderate to severe COVID-19, published in **Microorganisms**.

About RedHill Biopharma

RedHill Biopharma Ltd. (Nasdaq: RDHL) is a specialty biopharmaceutical company primarily focused on U.S. development and commercialization of drugs for gastrointestinal diseases, infectious diseases and oncology. RedHill promotes the FDA-approved gastrointestinal drug **Talicia®**, for the treatment of Helicobacter pylori (H. pylori) infection in adults[10], with a U.S. co-commercialization agreement with Cumberland Pharmaceuticals (Nasdaq: CPIX). RedHill's key clinical late-stage development programs include: (i) **opaganib** (ABC294640), a first-in-class, orally administered sphingosine kinase-2 (SPHK2) selective inhibitor with anti-inflammatory, antiviral, metabolic and anticancer activity, targeting multiple indications with U.S. government and academic collaborations intended for medical countermeasure development including for Ebola virus disease, radiation exposure indications such as GI-Acute Radiation Syndrome (GI-ARS), a Phase 2/3 program for hospitalized COVID-19, and an ongoing Phase 2 study in prostate cancer in combination with Bayer's darolutamide; (ii) **RHB-102** (Bekinda®), with a planned Phase 2 proof-of-concept study for GLP-1/GIP receptor agonist-associated GI intolerance, positive results from a U.S. Phase 3 study for acute gastroenteritis and gastritis, positive results from a U.S. Phase 2 study for IBS-D and potential UK submission for chemotherapy and radiotherapy induced nausea and vomiting. RHB-102 is partnered with Hyloris Pharmaceuticals (EBR: HYL) for worldwide development and commercialization outside North America; (iii) **RHB-204**, a next-generation optimized formulation of RHB-104, with a planned Phase 2 study for Crohn's disease (based on RHB-104's positive Phase 3 Crohn's disease study results); and (iv) **RHB-107** (upamostat), an oral broad-acting, host-directed, serine protease inhibitor with potential for pandemic preparedness, including COVID-19 and also targeting multiple cancer and inflammatory gastrointestinal diseases.

Visit **www.redhillbio.com** / **X.com/RedHillBio** for more information about the Company

Forward Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 and may discuss investment opportunities, stock analysis, financial performance, investor relations, and market trends. Such statements may be preceded by the words "intends," "may," "will," "plans," "expects," "anticipates," "projects," "predicts," "estimates," "aims," "believes," "hopes," "potential" or similar words, and include, among others, statements regarding the potential for the Company to succeed in its enforcement action against Kukbo and recovery any or all of the awards granted by the New York Supreme Court. 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 succeed in its enforcement action against Kukbo, and if successful may not recover all or any of awards granted by the New York Supreme Court; the risk that opaganib does not receive a priority review voucher, marketing exclusivity or accelerated development and review times; the risk that opaganib is not accepted into Ebola virus disease control programs, or if accepted, that it does not demonstrate efficacy; the risk that development of RHB-204 for Crohn's disease may not be completed, or if completed may not be approved or may not achieve commercial success; the risk that opaganib is not effective against the indications for which we develop our products; the risk that RHB-102 (Bekinda) does not effectively reduce GLP-1/GIP-related nausea, vomiting and diarrhea; the risk regarding the Company's ability to regain and maintain compliance with Nasdaq's listing requirements, including the minimum bid price requirement; the risk that the addition of new revenue generating products or out-licensing transactions will not occur; the risk that the Company will not receive future milestone payments under its existing agreements or that they will be less than anticipated; the risk of current uncertainty regarding U.S. government research and development funding and that the U.S. government is under no obligation to continue to support development of our products and can cease such support at any time; the risk that acceptance onto the RNCP Product Development Pipeline or other governmental and non-governmental development programs will not guarantee

ongoing development or that any such development will not be completed or successful; the risk that the FDA does not agree with the Company's proposed development plans for its programs; the risk that the Company's development programs and studies may not be successful and, even if successful, such studies and results may not be sufficient for regulatory applications, including emergency use or marketing applications, and that additional studies may be required; the risk that the Company will not successfully commercialize its products; 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 any necessary commercial companion diagnostics; (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; (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, 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) the Company's ability to collect on its judgment against Kukbo; (xiii) estimates of the Company's expenses, future revenues, capital requirements and needs for additional financing; (xiv) the effect of patients suffering adverse experiences using investigative drugs under the Company's Expanded Access Program; (xv) competition from other companies and technologies within the Company's industry; and (xvi) 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 April 27, 2026. 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
Chief Corporate & Business Development Officer
RedHill Biopharma
adi@redhillbio.com

Category: R&D

[1]Opaganib is an investigational new drug, not available for commercial distribution.

[2]**Abstract 7879: Opaganib in combination with oxaliplatin and doxorubicin as a novel salvage therapy for relapsed/refractory high-risk neuroblastoma**. **Jeremy Hengst**, **Mohammad Haque**, **Muhammad Younis**, **Thussenthan Walter Angelo**, **Anna Bourne**, **Katherine McClain**, **Meenakshi Shukla**, **Jonathan Lerch**, **Tarlan Arjmandi**, **Eric Cochran**, **Lynn Maines**, **Charles D. Smith**, **Vladimir S. Spiegelman**, **Jacqueline M. Kraveka**, **Giselle L. Saulnier Sholler**. Cancer Res (2026) 86 (7_Supplement): 7879. **https://doi.org/10.1158/1538-7445.AM2026-7879**

[3]Evaluate Ltd

[4]Neuenschwander FC, Barnett-Griness O, Piconi S, Maor Y, Sprinz E, Assy N, Khmelnitskiy O, Lomakin NV, Goloshchekin BM, Nahorecka E, et al. Effect of Opaganib on Supplemental Oxygen and Mortality in Patients with

Severe SARS-CoV-2 Based upon FIO2 Requirements. Microorganisms. 2024; 12(9):1767. **https://doi.org/10.3390/microorganisms12091767**

[5]**https://www.ncbi.nlm.nih.gov/books/NBK448111/#:~:text=Neuroblastoma%20is%20the%20most%20common,of%20pediatric%20cancer%2Drelated%20deaths**

[6]Yan P, Qi F, Bian L, et al. Comparison of Incidence and Outcomes of Neuroblastoma in Children, Adolescents, and Adults in the United States: A Surveillance, Epidemiology, and End Results (SEER) Program Population Study. Med Sci Monit. 2020;26:e927218. Published 2020 Nov 29. doi:10.12659/MSM.927218.

[7]**https://together.stjude.org/en-us/conditions/cancers/neuroblastoma.html**

[8]Flaadt T, Rehm J, Simon T, Hero B, Ladenstein RL, Lode HN, Grabow D, Nolte S, Crazzolara R, Greil J, Ebinger M, Abele M, Holzer U, Döring M, Schulte JH, Bader P, Schlegel PG, Eyrich M, Lang P, Klingebiel T, Handgretinger R. Long-Term Outcomes and Quality of Life of High-Risk Neuroblastoma Patients Treated with a Multimodal Treatment Including Anti-GD2 Immunotherapy: A Retrospective Cohort Study. Cancers (Basel). 2025 Jan 5;17(1):149. doi: 10.3390/cancers17010149. PMID: 39796776; PMCID: PMC11720496.

[9]**https://www.redhillbio.com/news/news-details/2025/RedHill-Announces-Initiation-of-Phase-2-Study-of-Opaganib-and-Darolutamide-in-Advanced-Prostate-Cancer/default.aspx**

[10]Talicia® (omeprazole magnesium, amoxicillin and rifabutin) is indicated for the treatment of H. pylori infection in adults. For full prescribing information see: **www.Talicia.com**.

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