# REGULUS THERAPEUTICS INC.

## FORM 8-K
(Current report filing)

Filed 06/12/17 for the Period Ending 06/09/17

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|---|---|
| Address | 10614 SCIENCE CENTER DRIVE<br>SAN DIEGO, CA 92121 |
| Telephone | 858-202-6300 |
| CIK | 0001505512 |
| Symbol | RGLS |
| SIC Code | 2834 - Pharmaceutical Preparations |
| Industry | Biotechnology & Medical Research |
| Sector | Healthcare |
| Fiscal Year | 12/31 |

# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION
**WASHINGTON, D.C. 20549**

# FORM 8-K

**CURRENT REPORT**
**Pursuant to Section 13 or 15(d)**
**of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): June 9, 2017**

# Regulus Therapeutics Inc.
**(Exact name of registrant as specified in its charter)**

| **Delaware** | **001-35670** | **26-4738379** |
|---|---|---|
| **(State of incorporation)** | **(Commission File No.)** | **(IRS Employer Identification No.)** |

| | |
|---|---|
| **10614 Sciences Center Drive**<br>**San Diego, CA** | **92121** |
| **(Address of principal executive offices)** | **(Zip Code)** |

**Registrant's telephone number, including area code: (858) 202-6300**

**N/A**
**(Former name or former address, if changed since last report.)**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☒

**Item 1.02 Termination of a Material Definitive Agreement.**

On June 9, 2017, AstraZeneca AB ("AstraZeneca") delivered written notice to Regulus Therapeutics Inc. (the "Company") of AstraZeneca's election to terminate the collaboration and license agreement entered into between AstraZeneca and the Company on August 5, 2012, as amended (the "Agreement"). In accordance with the Agreement, the termination will become effective on June 9, 2018, which is 12 months following the date of delivery of the notice by AstraZeneca.

Under the terms of the Agreement, the Company agreed to collaborate with AstraZeneca to identify, research and develop compounds targeting three microRNA alliance targets primarily in the fields of cardiovascular diseases, metabolic diseases and oncology. Pursuant to the Agreement, the Company granted AstraZeneca an exclusive, worldwide license to develop, manufacture and commercialize lead compounds designated by AstraZeneca in the course of the collaboration activities against the alliance targets for all human therapeutic uses. Under the terms of the Agreement the Company was required to use commercially reasonable efforts to perform all research, development and manufacturing activities described in the research plan, at the Company's cost, until the acceptance of an investigational new drug application or the end of the research term, which expired in August 2016. AZD4076(RG-125) was jointly identified and selected as a clinical candidate in April 2015 by AstraZeneca pursuant to the Agreement. Effective upon the termination of the Agreement, AstraZeneca's rights with respect to AZD4076(RG-125) will revert to the Company.

**Item 8.01 Other Events.**

On June 12, 2017, the Company announced in a press release the following updates with respect to the Company's pipeline.

**RG-012 for Alport syndrome** : Initiation of the Phase II clinical programs for RG-012 for the treatment of Alport syndrome is on track as planned. Important changes to the Phase II study design have been incorporated with the goal to accelerate patient enrollment, improve statistical power, and potentially achieve proof of mechanism data by the end of 2017. HERA, the Phase II randomized (1:1), double-blinded, placebo-controlled study evaluating the safety and efficacy of RG-012 in Alport syndrome patients, has been modified to increase enrollment to 40 patients to improve its statistical power. Dose frequency has also been adjusted to once every other week. The separate renal biopsy study will evaluate RG-012 renal tissue pharmacokinetics, target engagement and downstream effects on genomic disease biomarkers. Data from the renal biopsy study is anticipated by year-end and interim data from HERA is anticipated mid-2018.

**RG-101 (anti-miR122) for HCV:** The Company plans to discontinue clinical development of RG-101 upon completion of the one remaining clinical study, which is expected to occur in July 2017. Comprehensive pre-clinical investigation and thorough evaluation of the clinical data from RG-101 has led to the identification of a bilirubin transport mechanism as the likely cause for the cases of hyperbilirubinemia in the RG-101 program. The Company believes that a combination of factors including inhibition of conjugated bilirubin transport by RG-101, impaired baseline bilirubin transport in HCV patients and the preferential uptake of RG-101 by hepatocytes contributed to this mechanism. Additional patient specific contributing factors cannot be excluded. Applying the learnings from the RG-101 program, alternative compounds targeting miR-122 have been identified that maintain potent HCV antiviral activity while lacking inhibition of the bilirubin transporter. These compounds have the potential for rapid clinical proof-of-concept of a novel, markedly shortened treatment regimen for HCV and will be considered for further development pending an updated global commercial market assessment for HCV.

**RGLS4326 (anti-miR-17) for autosomal dominant polycystic kidney disease ( ADPKD):** The IND for RGLS4326 is on track for filing by year end 2017. IND enabling toxicology, repeat pharmacology and manufacturing work have been completed as scheduled to support regulatory submissions. Data from the pre-clinical program have been recently published in *Nature Communications* and support the rationale for targeting miR-17 for the treatment of ADPKD, an orphan indication with no treatment options affecting approximately 600,000 people in the United States.

**RGLS5040 (anti-miR-27) for cholestatic disease:** RGLS5040, an unconjugated inhibitor of microRNA27, has been discontinued based on a positioning of the compound with respect to the competitive landscape coupled with the results from repeat pharmacology studies as part of IND-enabling work. The Company continues to work on developing highly effective therapeutics for genetic forms of cholestatic disease as part of its overall research activities targeting unmet diseases of the liver and kidney.

**Forward-Looking Statements**

Statements contained in this Current Report on Form 8-K regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements associated with the expected ability of the Company to undertake certain activities and accomplish certain goals (including with respect to development and other activities related to RG-101 or RG-012), the projected timeline of clinical development activities, and expectations

regarding future therapeutic and commercial potential of the Company's business plans, technologies and intellectual property related to microRNA therapeutics and biomarkers being discovered and developed by the Company. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "intends," "will," "goal," "potential" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon the Company's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks associated with the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. These and other risks concerning the Company's financial position and programs are described in additional detail in the Company filings with the Securities and Exchange Commission, including under the caption "Risk Factors" in Regulus' Quarterly Report on Form 10-Q for the quarter ended March 31, 2017. All forward-looking statements contained in this Current Report on Form 8-K speak only as of the date on which they were made. The Company undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: June 12, 2017

Regulus Therapeutics Inc.

By: /s/ Joseph P. Hagan
Joseph P. Hagan
President and Chief Executive Officer