Regulus Therapeutics

Fact Sheet

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REGULUS THERAPEUTICS

QUICK REFERENCE

Regulus Therapeutics Inc.
Nasdaq: RGLS
www.REGULUSRX.com

BUSINESS SUMMARY

Regulus Therapeutics Inc. is a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs. Regulus has leveraged its oligonucleotide drug discovery and development expertise to develop a pipeline complemented by a rich intellectual property estate in the microRNA field.

TECHNOLOGY

We believe that targeting pathways of human disease with antisense oligonucleotides targeting RNA biology represents a novel and potentially powerful therapeutic approach for multiple diseases.

We are beginning this work with therapies designed to target microRNA regulation of protein expression. MicroRNAs are small, naturally occurring, non-coding RNAs that function as important regulators of gene expression of messenger RNA (mRNA) and play a role in multiple cellular processes. They do this by binding specific mRNA and blocking translation, leading to control of gene expression and direct degradation of target mRNA.

About ADPKD

Autosomal Dominant Polycystic Kidney Disease (ADPKD), caused by mutations in the PKD1 or PKD2 genes, is among the most common human monogenic disorders and a leading cause of end-stage renal disease. The disease is characterized by the development of multiple fluid filled cysts primarily in the kidneys, and to a lesser extent in the liver and other organs. Excessive kidney cyst cell proliferation, a central pathological feature, ultimately leads to end-stage renal disease in approximately 50% of ADPKD patients by age 60. Approximately 160,000 individuals are diagnosed with the disease in the United States alone, with an estimated global prevalence of 4 to 7 million.

About RGLS8429

RGLS8429 is a novel, next generation oligonucleotide for the treatment of ADPKD designed to inhibit miR-17 and to preferentially target the kidney. Administration of RGLS8429 has shown robust data in preclinical models, where clear improvements in kidney function, size, and other measures of disease severity have been demonstrated along with a superior pharmacologic profile in preclinical studies compared to Regulus' first-generation compound, RGLS4326. Regulus announced completion of the Phase 1 SAD study in September 2022. The Phase 1 SAD study demonstrated that RGLS8429 has a favorable safety and PK profile. RGLS8429 was well-tolerated with no serious adverse events reported and plasma exposure was approximately linear across the four doses tested and is similar to the PK data from the first-generation compound. In the Phase 1b MAD study Regulus announced both top line data from the first cohort of patients in September 2023 and from the second cohort of patients in March 2024. After review of all available safety data from the second cohort, Regulus has advanced to the third cohort where dosing has begun, and patients are receiving 3 mg/kg of RGLS8429 or placebo every other week for three months. Regulus announced completion of enrollment in the third cohort in January 2024 with top-line data anticipated in mid-2024.

CONTACT INFORMATION

Headquarters
4224 Campus Point Court, Suite 210
San Diego, CA 92121

Barwicki Investor Relations
Andrew Barwicki
516-662-9461 / andrew@barwicki.com

Recent Press Releases *(Headlines and Excerpts)*

Regulus Therapeutics Announces Completion of Enrollment in Third Cohort of Phase 1b Multiple-Ascending Dose (MAD) Clinical Trial of RGLS8429 for the Treatment of Patients with Autosomal Dominant Polycystic Kidney Disease (ADPKD)

Jan. 2, 2024 -- Regulus Therapeutics Inc. announced it has completed enrollment in the third cohort of patients in the Phase 1b MAD study of RGLS8429 for the treatment of ADPKD.

"The completion of our third and final randomized placebo-controlled cohort in the Phase 1b MAD study is an exciting step for Regulus, and we are happy to reach this stage of development for RGLS8429," said Jay Hagan, CEO of Regulus. "Our team is motivated and gearing up for 2024 when we expect topline data from our second cohort in Q1 2024, topline data from our third cohort in mid-2024, and the initiation of the fourth cohort, an open label fixed dose, of RGLS8429."

The Phase 1b MAD study is a double-blind, placebo-controlled trial evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics (PK/PD) of RGLS8429 in adult patients with ADPKD. The study will evaluate RGLS8429 treatment across three different weight-based dose levels, including measuring changes in polycystins, height-adjusted total kidney volume (htTKV), cyst architecture, and overall kidney function. The third cohort is being dosed at 3 mg/kg of RGLS8429 or placebo every other week for three months. The protocol was amended to include a fourth cohort of subjects who will receive an open label fixed dose of RGLS8429 to compare biomarker and safety data to the weight-based dosing.

Regulus Therapeutics Reports Third Quarter 2023 Financial Results and Recent Updates

- ***Announced positive topline data from the first cohort of patients in Phase 1b Multiple-Ascending Dose (MAD) Clinical Trial of RGLS8429 for the Treatment of Autosomal Dominant Polycystic Kidney Disease (ADPKD)***
- ***Completed enrollment in the second cohort of the Phase 1b MAD Clinical Trial of RGLS8429 for the Treatment of ADPKD***
- ***First patient dosed in the third cohort of the Phase 1b MAD Clinical Trial of RGLS8429 for the Treatment of ADPKD***

Nov. 9, 2023 -- Regulus Therapeutics Inc. today reported financial results and provided a corporate update for the third quarter ended September 30, 2023.

Program Updates

RGLS8429 for ADPKD: The Company held a virtual R&D Day on September 6, 2023, where the executive team joined academic leaders in ADPKD to discuss RGLS8429 along with the current unmet need in ADPKD, the role of genetics and polycystin in driving disease pathology, and the historical preclinical and clinical data that support targeting miR-17 as a therapeutic approach.

In September 2023, the Company announced the completion of enrollment of the second cohort in the Phase 1b MAD study of RGLS8429 for the treatment of ADPKD. The Phase 1b MAD study is a double-blind, placebo-controlled trial evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics of RGLS8429 in adult patients with ADPKD. The study will evaluate RGLS8429 treatment across three different dose levels, including measuring changes in polycystins, height-adjusted total kidney volume, cyst architecture, and overall kidney function. The second cohort is being dosed at 2 mg/kg of RGLS8429 or placebo every other week for three months.

The Company also shared positive topline data from the first cohort of patients in the phase 1b MAD study, in which patients were dosed at 1 mg/kg of RGLS8429 or placebo every other week for three months. Results showed increases in both PC1 and PC2 biomarkers. The mean PC1 level increase was statistically significant, with 36-41% increases at days 85 and 86 (n=9) compared to baseline. Numeric increases in PC2 levels were also observed, although not statistically significant.

In October 2023, the Company announced that, after a review of all available safety data in cohort two, it advanced to the third cohort in the Phase 1b MAD study. Shortly after, in November 2023, the Company announced dosing of the first patient in the third cohort. Patients in the third cohort are being dosed at 3mg/kg of RGLS8429 or placebo every other week for three months.

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Regulus Therapeutics Inc.
NASDAQ: RGLS

www.REGULUSRX.com

Barwicki Investor Relations * 30 Wall Street, 8 FL * New York, NY 10005

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