Rockwell Medical Announces Growth Plan and New Therapeutic Opportunities for Its Ferric Pyrophosphate Citrate (FPC) Platform

September 24, 2020

-New indications for FPC platform provide significant growth opportunity-

-Anticipates Type C meeting with FDA for home infusion in Q1 2021 and for acute heart failure in 2H 2021-

-Well capitalized to fund dialysis business and advance clinical development for home infusion indication-

-Archived webcast available on corporate website-

WIXOM, Mich., Sept. 24, 2020 (GLOBE NEWSWIRE) -- Rockwell Medical, Inc. (Nasdaq: RMTI) ("Rockwell Medical" or the "Company"), a biopharmaceutical company dedicated to transforming the treatment of iron deficiency and iron deficiency anemia management and improving outcomes for patients around the world, earlier today concluded its Management and Key Opinion Leader (KOL) conference call and webcast to discuss potential novel indications for its ferric pyrophosphate citrate (FPC) platform. The conference call included presentations by Rockwell Medical executive management and Connie Sullivan, B.S. Pharm., President and CEO of the National Home Infusion Association, and Inder Anand, M.D., F.R.C.P., D.Phil. (Oxon), Emeritus Professor of Medicine at the University of Minnesota Medical School and Former Director of the Heart Failure Program at VA Medical Center in Minneapolis.

"Earlier today, Rockwell Medical and independent leading experts laid out the pathway for potential important value creation for the Company through the development of the FPC platform to treat medical conditions with unmet clinical needs outside of the hemodialysis setting. This exciting new development plan for FPC, along with increasing adoption of Triferic for dialysis patients, are evidence of Rockwell Medical's continued focus on our vision – to transform the treatment of iron deficiency and anemia for the millions of people affected worldwide," said Russell Ellison, M.D., M.Sc., President and Chief Executive Officer of Rockwell Medical. "FPC for the treatment of iron deficiency anemia (IDA) in home infusion patients is our top development priority, which we view as a tractable clinical development project with relatively low safety risk given our extensive safety database of FPC, and low efficacy risk given our expertise and experience in the use of parenteral iron for the treatment of IDA. We expect to hold a Type C meeting with the U.S. Food and Drug Administration (FDA) in the first quarter of 2021 to discuss the program further. We are well capitalized to fund our existing dialysis business and advance clinical development for a home infusion indication, and we expect opportunities for multiple data read-outs and milestone reports from the home infusion program over the next three years as we advance the program through Phase II clinical proof-of-concept development."

Dr. Ellison added: "In addition, we discussed potentially pursuing an indication for FPC in hospitalized patients with acute heart failure. We believe FPC could be uniquely suited for these patients, based upon a completed mechanistic proof-of-concept study and the immediately bioavailable iron that FPC can deliver to patients during a short hospital stay. We continue to evaluate this opportunity and anticipate holding a Type C meeting with the FDA in the second half of 2021 to discuss the pathway for a potential clinical development program."

Highlights from the conference call and webcast are as follows:

Home Infusion

- Home infusion therapy is a rapidly growing area of medicine, with over 3.2 million patients served in 2019. We believe the growth trend is likely to continue driven by cost savings versus office-based or hospital care, an improving reimbursement landscape, and emerging standards resulting from the COVID-19 pandemic.
- IDA is a common co-morbidity in many sub-groups of patients receiving home infusion therapy, particularly in those receiving long-term home parenteral nutrition (HPN).
- Management of IDA in home infusion patients is currently a 'broken' process, according to the National Home Infusion Association, due to limitations with currently available parenteral iron products and other factors.
- FPC is uniquely suited for the treatment of IDA in the home infusion population based upon its kinetic profile and outstanding safety profile in both clinical trials and post-marketing use, and may fill an unmet clinical need as a uniquely suitable home infusion therapy for treatment of IDA.
- Home infusion therapy patients with IDA may be candidates for treatment with FPC across additional therapeutic categories besides HPN. These categories include antineoplastic chemotherapy, hydration therapy, inotropics and biologics. The U.S. market opportunity for HPN is estimated to be $200 million per year, and the U.S. market opportunity for the other categories above is estimated to be $400 million per year.
- The majority of home infusion therapy patients are covered by commercial insurance, and payers are increasingly motivated to reimburse home infusions to reduce in-office visits and hospitalizations, and save costs.
- Rockwell Medical intends to hold a Type C meeting with the FDA in Q1 2021 to discuss the pathway for clinical development. The Company anticipates conducting a Phase II observational study and a dose scheduling study to achieve proof-of-concept, with the goal of ultimately securing a broad label claim of treatment of IDA in adult patients receiving home infusion therapy regardless of etiology.

- The Company expects Phase II data around the end of 2021.

Acute Heart Failure

- Heart failure (HF) in the U.S. is a large and growing patient population. More than one million patients are hospitalized each year with acute decompensated heart failure, and iron deficiency (ID) is a common co-morbidity in all forms of HF.
- The acute heart failure (AHF) opportunity for FPC is associated with a large patient population with a high incidence of iron deficiency. FPC has theoretical acute clinical advantages (<30 days) vs. traditional IV iron therapy for iron deficient acute heart failure patients and could have an impact on hospital length of stay and/or 30-day readmissions.
- The majority of heart failure patients admitted to the hospital are insured by Medicare/Medicaid through a well-established universal approach usually resulting in a bundled reimbursement structure.
- Rockwell Medical intends to hold a Type C meeting with the FDA in the second half of 2021 to discuss a potential regulatory pathway. This meeting with the FDA will inform whether the Company ultimately pursues an indication in AHF.

An archived webcast of the call will be available under “Events & Presentations” in the Investor section of the Company’s website, https://ir.rockwellmed.com/.

About Rockwell Medical

Rockwell Medical is a biopharmaceutical company dedicated to transforming anemia management in a wide variety of therapeutic areas and across the globe, improving the lives of very sick patients. The Company’s initial focus is the treatment of anemia in end-stage kidney disease (ESKD). Rockwell Medical’s exclusive renal drug therapies, Triferic (ferric pyrophosphate citrate) Dialysate and Triferic AVNU (ferric pyrophosphate citrate injection), are the only FDA-approved therapeutics indicated for iron replacement and maintenance of hemoglobin in hemodialysis patients. Rockwell Medical is also an established manufacturer, supplier and leader in delivering high-quality hemodialysis concentrates/dialysates to dialysis providers and distributors in the U.S. and abroad.

About Triferic Dialysate and Triferic AVNU

Triferic Dialysate and Triferic AVNU are the only FDA-approved therapies in the U.S. indicated to replace iron and maintain hemoglobin in hemodialysis patients during each dialysis treatment. Triferic Dialysate and Triferic AVNU have a unique and differentiated mechanism of action, which has the potential to benefit patients and health care economics. Triferic Dialysate and Triferic AVNU represent a potential innovative medical advancement in hemodialysis patient iron management – with the potential to become the future standard of care.

Triferic Dialysate and Triferic AVNU both deliver approximately 5-7 mg iron with every hemodialysis treatment to the bone marrow and maintain hemoglobin without increasing iron stores (ferritin). Both formulations donate iron immediately and completely to transferrin (carrier of iron in the body) upon entry into the blood which is then transported directly to the bone marrow to be incorporated into hemoglobin, with no increase in ferritin (stored iron and inflammation) and no reports of anaphylaxis in over 1,000,000 patient administrations, addressing a significant medical need in overcoming Functional Iron Deficiency (FID) in ESKD patients.

Important Safety Information

Serious hypersensitivity reactions, including anaphylactic-type reactions, some of which have been life-threatening and fatal, have been reported in patients receiving parenteral iron products. Patients may present with shock, clinically significant hypotension, loss of consciousness, and/or collapse. Monitor patients for signs and symptoms of hypersensitivity during and after hemodialysis until clinically stable. Personnel and therapies should be immediately available for the treatment of serious hypersensitivity reactions. Hypersensitivity reactions have been reported in 1 (0.3%) of 292 patients receiving Triferic in two randomized clinical trials.

Iron status should be determined on pre-dialysis blood samples. Post dialysis serum iron parameters may overestimate serum iron and transferrin saturation.

The most common adverse reactions (≥3% and at least 1% greater than placebo) in controlled clinical studies include: procedural hypotension (21.6%), muscle spasms (9.6%), headache (9.2%), pain in extremity (6.8%), peripheral edema (6.8%), dyspnea (5.8%), back pain (4.5%), pyrexia (4.5%), urinary tract infection (4.5%), asthenia (4.1%), fatigue (3.8%), arteriovenous (AV) fistula thrombosis (3.4%), and AV fistula site hemorrhage (3.4%).

Triferic® is a registered trademark of Rockwell Medical, Inc.

Forward Looking Statements

Certain statements in this press release may constitute “forward-looking statements” within the meaning of the federal securities laws, including, but not limited to, potential new indication opportunities for Ferric Pyrophosphate Citrate (“FPC”), the potential for FPC to address the significant unmet need for home infusion patients, market opportunities for new indications, the clinical risk and safety profiles of new indications, the development of a clinical plan and timing of FDA review of FPC for new indications, the potential for reimbursement of FPC for new indications, the growth of the home and specialty infusion marketplace, the timing and success of regulatory filings for new indications, the timing and cost of clinical development plans and clinical study designs. Words such as, “may,” “might,” “will,” “should,” “believe,” “expect,” “anticipate,” “estimate,” “continue,” “could,” “can,” “would,” “develop,” “plan,” “potential,” “predict,” “forecast,” “project,” “intend” or the negative of these terms, and similar expressions, or statements regarding intent, belief, or current expectations, are forward looking statements. While Rockwell Medical believes these forward-looking statements are reasonable, undue reliance should not be placed on any such forward-looking statements, which are based on information available to us on the date of this release. These forward-looking statements are based upon current estimates and assumptions and are subject to various risks and uncertainties (including, without limitation, those set forth in Rockwell Medical’s SEC filings), many of which are beyond our control and subject to change. Actual results could be materially different. Risks and uncertainties include, but are not limited to: the impact of the COVID-19 pandemic (including, applicable federal, state or local orders) on business and clinical development plans; the risk that regulatory authorities delay or fail to approve FPC for new indications; the risk that market opportunities are smaller than estimated; the risk that Rockwell Medical is not able to seek

reimbursement for FPC for new indications; the risk that FPC is unsafe for new indications; the risk that clinical study designs, timing and costs are different than estimated; and those risks more fully discussed in the “Risk Factors” section of our Quarterly Report on Form 10-Q for the period ended June 30, 2020, and of our Annual Report on Form 10-K for the year ended December 31, 2019, as such description may be amended or updated in any future reports we file with the SEC. Rockwell Medical expressly disclaims any obligation to update our forward-looking statements, except as may be required by law.

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