CORRECTION: Radius Health Announces Plans for Global Prader-Willi Syndrome Pivotal Study

July 23, 2021

- FDA Type C meeting minutes provide clarity on the Phase 2/3 study design for RAD011
- 505(b)(2) regulatory strategy: acceptable approach upon completion of PK & food effect studies
- Based on results, a single well controlled study could serve as the basis for marketing approval
- RAD011 had previously been granted Orphan Drug and Fast Track Designation by the FDA
- Study initiation targeted by end of year or early first quarter of 2022

BOSTON, July 23, 2021 (GLOBE NEWSWIRE) -- A correction has been issued for a release disseminated under the same headline on July 22nd at 9:00 am EST by Radius Health, Inc. (RDUS), please note that the corrected release contains the revised statement with the typographical correction as follows: “RAD011 is not scheduled as it does not have traceable amounts of THC,” which has been corrected from “does have.” This information is located in the last sentence of the last paragraph of the main body of the press release.

Radius Health, Inc. (“Radius” or the “Company”) (NASDAQ: RDUS) today announced that it has recently received the written meeting minutes from a June Type C meeting held with the U.S. Food and Drug Administration (“FDA”) regarding RAD011, a synthetic cannabidiol oral solution.

RAD011 is initially to be utilized for the treatment of Prader-Willi Syndrome (“PWS”). RAD011 has previously been granted Orphan Drug and Fast Track Designation by the FDA. Based on the feedback received, Radius plans on initiating a pivotal Phase 2/3 global study for patients with PWS.

The main highlights from the FDA meeting minutes are set out below:

- Design characteristics and endpoints for a single seamless, pivotal Phase 2/3 study for PWS
- Results dependent, a single adequate and well-controlled study could serve as the basis for marketing approval
- Acceptability of 505(b)(2) regulatory pathway subject to completion and review of PK bridging and dedicated food effect studies
- Acceptability of nonclinical package to support the pivotal Phase 2/3 study

Paige Rivard, CEO of Prader-Willi Syndrome Association USA said, “We are encouraged by Radius’ commitment to advance RAD011 for the treatment of debilitating symptoms associated with PWS, particularly hyperphagia. We look forward to supporting their team throughout their study by raising awareness of their planned study with key opinion leaders, caregivers and individuals within the PWS community, and providing a means to gather perspective of individuals with PWS and critical caregivers.”

Phase 2/3 Study
Radius plans to initiate the pivotal Phase 2/3 study, to be branded as “SCOUT” (**S**ynthetic **C**annabidiol **O**ral Sol**ut**ion), by end of this year or early first quarter of 2022. With this current initiation timeline, it is anticipated top line results would be available in the second half of 2024.

The proposed study parameters, informed by several global advisory board meetings completed with leading KOLs, PWS advocacy organizations, and feedback from the FDA, are highlighted below:

- The pivotal Phase 2/3 study (SCOUT-015) will be a double-blind, placebo-controlled, seamless pivotal Phase 2/3 study in individuals with genetically-confirmed PWS, ages 8 to 65
- The seamless design will evaluate safety and tolerability across multiple dose groups in the Phase 2 portion, narrow the dose selection for the Phase 3 portion, and anticipated to enable one study to evaluate efficacy, safety and tolerability
- A screening and placebo lead-in period will precede the 26-week maintenance period
- Approximately 200 PWS individuals at 30+ global sites are planned for inclusion in SCOUT-015
- Primary endpoint: change in Hyperphagia Questionnaire for Clinical Trials (HQ-CT) from baseline
- Eligible individuals have the option to enroll in a long-term extension safety study (SCOUT-016)

Liz Messersmith, Ph.D. in Neuroscience and Senior Vice President at Radius stated, “We have established an exceptionally strong and highly experienced team of dedicated clinical, medical, biometrics, advocacy, regulatory and CMC talent to execute the pivotal study for PWS.”

Dr. Messersmith added further that, “We intend to use PWS as the anchor indication for RAD011. Additional disease opportunities and clinical trial initiatives will be shared in due course.”

Strategic CRO Partner Selected & DEA Scheduling Guidance Received
The Company is working closely with CTI Clinical Trial & Consulting, a world-leading CRO in rare and orphan disease research, to initiate and execute SCOUT-015 and SCOUT-016.

Important to the initiation and execution of the SCOUT program, Radius will move forward with RAD011 as not scheduled under the Controlled Substance Act (“CSA”) based on guidance from the Drug Enforcement Administration (“DEA”). The guidance states if a product does not contain any quantity of synthetic THC (or any other controlled substance), it is not controlled under the CSA. RAD011 is not scheduled as it does not have traceable amounts of THC.

About Prader-Willi Syndrome
PWS, an orphan disease, is a complex genetic disorder with clinical manifestations on the endocrine and neurological systems. Clinical signs of PWS develop throughout childhood, with hyperphagia and anxiety ranked as the key clinical features seeking medical attention by caregivers of individuals with PWS. Hyperphagia is a relentless, insatiable, pathological drive to eat that requires caregivers to strictly manage access to food through the locking of cabinets and refrigerators. PWS is recognized as the leading genetic cause of life-threatening obesity in children. As life-threatening hyperphagia persists into adulthood, metabolic syndrome expressed through obesity and diabetes can develop and contribute to morbidity and mortality. In addition to food-related behaviors, the behavioral symptoms commonly observed in PWS include high irritability, habitual skin picking, oppositional defiance and cognitive rigidity. There are currently no approved therapies to treat this disorder's hyperphagia, irritability, or metabolic aspects. In the U.S., PWS occurs in approximately one out of every 15,000 births.

About RAD011
Investigational drug RAD011 is a pharmaceutical-grade synthetic cannabidiol oral solution, manufactured utilizing traditional pharmaceutical manufacturing processes. The product has purity specifications that meet standardized regulatory and quality control requirements and, compared to the process of developing a plant-derived product, the synthetic manufacturing process usually enables increased consistency and greater precision in the product supply. RAD011 has been assessed in over 150 patients across multiple indications and has potential utilization in multiple endocrine and metabolic orphan diseases. Radius is initially targeting Prader-Willi syndrome (PWS) and anticipates initiating a seamless pivotal Phase 2/3 study for patients with PWS in the second half of 2021.

About Radius
Radius is a commercial biopharmaceutical company committed to serving patients with unmet medical needs in endocrinology and other therapeutic areas. Radius' lead product, TYMLOS® (abaloparatide) injection, was approved by the U.S. Food and Drug Administration for the treatment of postmenopausal women with osteoporosis at high risk for fracture. The Radius clinical pipeline includes investigational abaloparatide injection for potential use in the treatment of men with osteoporosis; an investigational abaloparatide transdermal system for potential use in the treatment of postmenopausal women with osteoporosis; the investigational drug, elacestrant (RAD1901), for potential use in the treatment of hormone-receptor positive breast cancer out-licensed to Menarini Group; and the investigational drug RAD011, a synthetic cannabidiol oral solution with potential utilization in multiple endocrine and metabolic orphan diseases, initially targeting Prader-Willi syndrome.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation statements regarding our expectations with respect to our SCOUT-15 and SCOUT-16 clinical trials and timing of receipt of topline results therefrom.

These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the following: the adverse impact the ongoing COVID-19 pandemic is having and is expected to continue to have on our business, financial condition and results of operations, including our commercial operations and sales, clinical trials, preclinical studies, and employees; quarterly fluctuation in our financial results; our dependence on the success of TYMLOS, and our inability to ensure that TYMLOS will obtain regulatory approval outside the U.S. or be successfully commercialized in any market in which it is approved, including as a result of risk related to coverage, pricing and reimbursement; risks related to competitive products; risks related to our ability to successfully enter into collaboration, partnership, license or similar agreements; risks related to clinical trials, including our reliance on third parties to conduct key portions of our clinical trials and uncertainty that the results of those trials will support our product candidate claims; the risk that adverse side effects will be identified during the development of our product candidates or during commercialization, if approved; risks related to manufacturing, supply and distribution; and the risk of litigation or other challenges regarding our intellectual property rights. These and other important risks and uncertainties discussed in our filings with the Securities and Exchange Commission, or SEC, including under the caption "Risk Factors" in our Annual Report on Form 10-K for the year ending December 31, 2020 and subsequent filings with the SEC, could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, we disclaim any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

Investor & Media Relations Contact:
Ethan Holdaway
Email: investor-relations@radiuspharm.com
Phone: (617) 583-2017

Source: Radius Health Inc.