Bird Rock Bio Submits Clinical Trial Authorization for the First in Human Clinical Trial for Namacizumab

- ***Namacizumab is a first-in-class and only-in-class negative allosteric modulating antibody (NAMA) that stabilizes the cannabinoid 1 receptor (CB1) in an inactive conformation.***
- ***Namacizumab is being developed to treat large unmet medical needs in fibrotic and metabolic disease, including non-alcoholic steatohepatitis (NASH) and diabetic nephropathy.***
- ***The double blind, placebo controlled, dose ranging Phase 1 clinical trial will include a Single Ascending Dose Study in healthy volunteers and a Multiple Ascending Dose Study in non-alcoholic fatty liver disease (NAFLD) patients, assessing outcomes of key biomarkers.***
- ***The results are anticipated by Q4 2017 and are expected to support later stage clinical trials in multiple indications.***
- ***Namacizumab was discovered internally through Bird Rock Bio's proprietary iCAPS platform, which is specialized to enable the discovery of functional antibodies to G protein coupled receptors.***

La Jolla, CA, October 27, 2016 -- Bird Rock Bio, Inc., a clinical stage biopharmaceutical company, announced that today it has requested approval for the initiation of a two-part Phase 1 clinical trial for namacizumab, a novel therapeutic antibody to the cannabinoid 1 receptor (CB1). The trial is anticipated to provide important safety, tolerability and biomarker efficacy data for namacizumab to support differentiated clinical potential in fibrotic and metabolic disease. As a first-in-class and only-in-class negative allosteric modulating antibody (NAMA) that stabilizes the inactive conformation of CB1, namacizumab has the potential to build on the significant historic mechanistic and clinical data on the modulation of CB1 in disease.

According to **Paul Grayson, Bird Rock Bio's CEO**, "2016 has been a year of exceptional progress for our Company. This submission for the first in human clinical trial for namacizumab represents the culmination of four years of extraordinary efforts by our scientific and development team. Achievement of this milestone for a novel and highly differentiated biologic that can address several of the world's largest emerging medical needs provides tremendous opportunity and excitement for our organization, partners and investors. "

Namacizumab's Unique Position as the Only Therapeutic Antibody to CB1.
CB1 modulation has been extensively researched pre-clinically and clinically by many of the largest pharmaceutical companies in metabolic and fibrotic diseases and has demonstrated substantial efficacy potential. Clinical development for small molecule CB1 modulators had been largely abandoned due to the emergence of adverse effects that were associated with their penetration of the central nervous system (CNS). As antibodies are large molecule proteins that typically do not penetrate the blood brain barrier, and thus are expected to avoid the CNS, Bird Rock Bio selected CB1 as a top target for its antibody discovery.

Following the groundbreaking discovery as the only known negative allosteric modulating antibody to CB1, namacizumab has been evaluated in multiple preclinical safety studies which have confirmed the antibody to be restricted to peripheral tissues and avoidance of the CNS. In a large IND-enabling toxicology study recently completed, as much as 75 times the anticipated therapeutic dose of namacizumab was administered with no significant adverse effects. These favorable outcomes have been a key part of the advancement of namacizumab into clinical development.

Namacizumab Phase 1 Clinical Trial.
A single ascending dose (SAD) trial in 24 healthy volunteers will assess safety, tolerability, and pharmacokinetics. A multiple ascending dose (MAD) trial will be initiated following the safety review of the last SAD cohort and will include up to 60 non-alcoholic fatty liver disease (NAFLD) patients. Multiple clinically validated biomarkers such as de novo lipogenesis, liver fat and glucose tolerance will be assessed in the MAD trial. Data from the SAD and MAD are anticipated to be available in Q1 2017 and Q4 2017 respectively.

The trial was designed in collaboration with Bird Rock Bio's clinical advisory board, which includes leading NASH experts, Scott Friedman, MD (Chief, Division of Liver Diseases, Icahn School of Medicine at Mount Sinai), Rohit Loomba, MD (Professor of Medicine (with tenure) in the Division of Gastroenterology, and Adjunct Professor in the Division of Epidemiology at University of California, San Diego) and Stephen Harrison, MD LTC (Chief of Hepatology at Brooke Army Medical Center, Fort Sam Houston, TX).

According to Dr. Scott L. Friedman: "with its potential role in the metabolic, inflammatory, and fibrotic components of NASH, namacizumab is among the first drug candidates to address these 3 key features of the disease that drive clinical progression. As a monoclonal antibody, namacizumab has been shown in multiple pre-clinical studies to be safe and well tolerated and to not enter the central nervous system. There is currently no FDA approved treatment for NASH, a growing epidemic affecting up to 15 million people in the US and expected to become the leading cause of liver transplant by 2020."

Additionally, NASH patients are at high risk of morbidity and mortality from cardiovascular comorbid disease. Unlike many of the alternative compounds currently in clinical development for NASH, namacizumab has the potential to favorably effect multiple cardiovascular and metabolic risk factors. Combined with its multimodal mechanistic profile, this potential safety enhancement is anticipated to be a strong competitive differentiator for namacizumab.

From a manufacturing standpoint, namacizumab has been efficiently produced in CHO-K1 cells, demonstrating favorable expression of approximately 2.8 grams per liter. Accordingly, manufacturing of clinical supply is complete for both the Phase 1a and Phase 1b trials (more than 2,100 vials of drug product) and is well positioned for late stage development and commercial needs.

"Having accomplished numerous critical development goals for namacizumab, Bird Rock Bio is well positioned to achieve major clinical milestones in 2017," said **Paul Grayson**. "We look forward to providing additional updates in the near future."

About Namacizumab

Discovered internally through Bird Rock Bio's proprietary iCAPS platform, namacizumab is scheduled to be the first and only known NAMA to the CB1 receptor to be entering clinical trials. Namacizumab is a multi-modal therapeutic candidate with fibrotic, inflammatory and metabolic mechanisms of action. This provides for the opportunity for namacizumab to have significant potential across a broad range of fibrotic and metabolic diseases including large unmet medical conditions such as NASH and diabetic nephropathy.

About iCAPS

Bird Rock Bio's iCAPS platform, the leading GPCR allosteric antibody drug discovery platform, can isolate and present functional GPCRs in the correct confirmation to identify selective monoclonal antibody allosteric modulators. GPCRs are a valuable class of drug targets but have been largely unexplored in antibody discovery because of the difficulty in isolating GPCRs in the correct conformation and functional form.

About Bird Rock Bio, Inc.

Backed by leading biotechnology venture investors, Bird Rock Bio's strategy leverages biologic targets with substantial human proof of mechanism for the development of first in class or best in class molecules with strong clinical and commercial differentiation. The Company is focused on developing proprietary antibodies for fibrotic, metabolic and inflammatory diseases, including RA, SA, NASH and diabetic nephropathy. Bird Rock Bio's science team is experienced with translating pioneering research into promising therapeutics with potential deep pharmacoeconomic benefits. For more, visit www.birdrockbio.com.

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