



JANUARY 22, 2020 (HTTP://ARUVANT.COM/2020/01/22/)

## Aruvant Receives Orphan Drug Designation for ARU-1801 for the Treatment of Sickle Cell Disease

- ARU-1801 is being developed as a potential one-time treatment for patients suffering from sickle cell disease and  $\beta$ -thalassemia
- ARU-1801 has been designed to deliver a highly potent gene payload with enhanced anti-sickling properties, allowing for a Reduced Intensity Conditioning (RIC) regimen

NEW YORK and BASEL, Switzerland, Jan. 22, 2020 — Aruvant, a clinical-stage biopharmaceutical company focused on developing and commercializing transformative therapies for the treatment of severe blood disorders, today announced that the U.S. Food and Drug Administration has granted Orphan Drug designation to ARU-1801, Aruvant's investigational therapy for the treatment of sickle cell disease.

The FDA grants Orphan Drug designation to novel therapies that treat diseases impacting fewer than 200,000 individuals in the United States. The benefits of orphan designation include a seven-year period of marketing exclusivity in the U.S. upon approval if received, waiver of certain FDA user-fees, and tax credits for qualified clinical trials.

“We are excited to build on the momentum afforded by both today's announcement of Orphan Drug status as well as our recent announcement of Rare Pediatric Disease status for ARU-1801,” said Will Chou, M.D., Chief Executive Officer of Aruvant. “For patients suffering from sickle cell disease, we believe the ultimate promise of gene therapy is a one-time cure without the side effect profile of high intensity myeloablative conditioning. We are committed to providing patients with that option and look forward to presenting more data on our Reduced Intensity Conditioning (RIC) approach as patients continue to be treated in our ongoing Phase 1/2 study.”

### About Sickle Cell Disease and $\beta$ -Thalassemia

Sickle cell disease is a progressively debilitating and life-threatening inherited red blood cell disorder that causes a patient's oxygen-carrying cells to be abnormally inflexible and sickle-shaped. This genetic defect leads to lowered oxygen-carrying capacity in the



hemoglobin protein of these red blood cells. Sickle cell disease can cause inflammation of extremities, bacterial infections, stroke, anemia, and attacks of pain called sickle cell crises.

$\beta$ -thalassemia is an inherited red blood cell disorder distinguished by reduced or nonexistent production of functional  $\beta$ -globin, a critical component of adult hemoglobin. Patients with the disorder suffer from anemia, which can cause weakness, fatigue, and more serious complications. Individuals with  $\beta$ -thalassemia are at an increased risk of developing abnormal blood clots.

## About Aruvant

Aruvant Sciences, part of the Roivant family of companies, is a clinical-stage biopharmaceutical company focused on developing and commercializing transformative therapies for the treatment of severe blood disorders, with an emphasis on helping patients suffering from sickle cell disease and  $\beta$ -thalassemia. The company's lead candidate, ARU-1801, is a modified fetal hemoglobin gene therapy for sickle cell disease and related hemoglobinopathies. For more information, please visit [www.aruvant.com](http://www.aruvant.com) ([https://protect-us.mimecast.com/s/KUIsCjRgEJfnBAGOUGOcU\\_?domain=roivant.us13.list-manage.com](https://protect-us.mimecast.com/s/KUIsCjRgEJfnBAGOUGOcU_?domain=roivant.us13.list-manage.com)).

## About Roivant

Roivant aims to improve health by rapidly delivering innovative medicines and technologies to patients. Roivant does this by building Vants – nimble, entrepreneurial biotech and healthcare technology companies with a unique approach to sourcing talent, aligning incentives, and deploying technology to drive greater efficiency in R&D and commercialization. For more information, please visit [www.roivant.com](http://www.roivant.com) (<https://protect-us.mimecast.com/s/pnpACkRjGMfOK4Xwi4zkXK?domain=roivant.us13.list-manage.com>).

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**ARUVANT RECEIVES FDA RARE PEDIATRIC DISEASE  
DESIGNATION FOR ARU-1801 FOR THE TREATMENT OF**  
(<http://aruvant.com/aruvant-receives-fda-rare-pediatric-disease-designation-for-aruvant-1801-for-the-treatment-of-sickle-cell-disease/>)



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