Sanofi builds focus on rare blood disorders and cancers

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Some of the most serious unmet patient needs today are in the field of hematology. Rare blood disorders and blood-related cancers continue to be a major focus of research as scientists look for new treatments for serious and life-threatening conditions.

To help meet this need, Sanofi has significantly increased its focus on hematology, making a number of important investments to advance new therapies and improve the care of patients affected by rare diseases, hemophilia, acquired thrombotic thrombocytopenic purpura (aTTP), cold agglutinin disease and other rare blood disorders.

The company’s progress will be evident at the 60th American Society of Hematology (ASH) Annual Meeting and Exposition being held December 1 - 4 in San Diego, CA.

“Hematology has become a major focus for Sanofi, and we are very excited about the progress we are making in advancing research on a relatively broad range of potential new treatments for people with rare blood disorders and blood cancers,” said Bill Sibold, Executive Vice President and Head of Sanofi Genzyme, the specialty care global business unit of Sanofi. “We look forward to sharing that progress with the hematology community at this year’s ASH meeting.”

Advancements in Cancer (Hematological Malignancies)

Data presented at ASH include the latest research regarding isatuximab, an anti-CD38 monoclonal antibody in late-stage development for patients with multiple myeloma, a rare plasma cell malignancy with high unmet medical need. Isatuximab is currently being investigated in four Phase 3 clinical trials, including three company sponsored pivotal trials.

Multiple Myeloma Oral Presentations at ASH:

- Results from a Phase II Study of Isatuximab As a Single Agent and in Combination with Dexamethasone in Patients with Relapsed/Refractory Multiple Myeloma (abstract 155; oral presentation on Saturday, December 1, 2018: 12:00-1:30 PM (PT))
- Preliminary Results from a Phase I Study of Isatuximab (ISA) in Combination with Bortezomib, Lenalidomide, Dexamethasone (VRd), and in Patients with Newly Diagnosed Multiple Myeloma (NDMM) Non-Eligible for Transplant (abstract 595; oral presentation on Monday, December 3, 2018: 7:00-8:30 AM (PT))

Advancements in Rare Blood Disorders

Sanofi’s increased commitment to the rare blood disorders community follows the acquisition of two companies, Bioverativ and Ablynx, earlier this year. Sanofi will create a Rare Blood Disorders franchise in early 2019 that will become part of Sanofi Genzyme. In addition to two marketed therapies for the treatment of hemophilia, Sanofi’s pipeline includes novel investigational therapies in hemophilia, sickle cell disease\(^1\), cold agglutinin disease and aTTP, a rare blood-clotting disorder.

Key Hemophilia Presentations at ASH:

- BVV001: The First Investigational Factor VII Therapy to Break Through the VWF Ceiling in Hemophilia A with Potential for Extended Protection for One Week or Longer (abstract 636; oral presentation on Monday, December 3, 2018: 7:00-8:00 PM (PT))\(^2\)
- ASPIRE Final Results Confirm Established Safety, Sustained Efficacy and Extended Dosing Interval for Up to 4 Years of Treatment With rFVIIIFc in Previously Treated Subjects With Severe Hemophilia A (abstract 1192; poster on Saturday, December 1, 2018: 6:15-8:15 PM (PT))\(^3\)
- B-YOND Final Results Confirm Established Safety, Sustained Efficacy, and Extended Dosing Interval for Up to 4 Years of Treatment With rFVIIIFc in Previously Treated Subjects With Severe Hemophilia B (abstract 1214; poster on Saturday, December 1, 2018: 6:15-8:15 PM (PT))\(^3\)

aTTP Oral Presentation at ASH:

- Integrated Efficacy Results from the Phase II and Phase III Randomized Studies with Caplacizumab in Patients with Acquired Thrombotic Thrombocytopenic Purpura (abstract number 373; oral presentation on Sunday, December 2, 2018: 12:00-1:30 PM (PT))

Advancements in Rare Genetic Conditions

Sanofi Genzyme has worked in rare genetic conditions, including Gaucher disease for more than 30 years. Gaucher disease is an inherited genetic condition that can affect the blood, organs and bones. Because Gaucher disease has similar signs and symptoms of some more common hematologic malignancies, it is often misdiagnosed. Data presented at ASH includes oral elaglustat for the treatment of Gaucher disease type 1.

Gaucher Disease Presentations at ASH:

- Long-Term Effects of Oral Elaglustat on Skeletal Manifestations of Gaucher Disease Type 1: Results from Four Completed Clinical Trials (abstract 2396; poster on Sunday, December 2, 6:00-8:00 PM (PT))
- The Long-Term Adverse Event Profile of Oral Elaglustat for the Treatment of Gaucher Disease Type 1: Pooled Analysis of Data from 393 Patients in 4 Completed Trials (abstract 3692; poster on Monday, December 3, 6:00-8:00 PM (PT))

Isatuximab and BVV001 are investigational agents and have not been approved by the U.S. Food and Drug Administration (FDA) or any other regulatory agency worldwide for the use under investigation.

The U.S. FDA has accepted for priority review the Biologics License Application for caplacizumab for treatment of patients 18 years of age and older experiencing an episode of aTTP. The European Commission approved caplacizumab as the first therapeutic specifically indicated for the treatment of aTTP earlier this year.
Bioverativ, a Sanofi company, and Sangamo Therapeutics, Inc. have an exclusive worldwide collaboration to develop and commercialize ZFN-mediated gene-edited cell therapies for the treatment of beta thalassemia and sickle cell disease.

Bioverativ, a Sanofi company, is responsible for the development of BIVV001.

Bioverativ, a Sanofi company, and Sobi collaborate on the development and commercialization of rFVIIIFc and rFIXFc.

CERDELGA® (eliglustat)

Indications & Usage

CERDELGA is indicated for the long-term treatment of adult patients with Gaucher disease type 1 (GD1) who are CYP2D6 extensive metabolizers (EMs), intermediate metabolizers (IMs), or poor metabolizers (PMs) as detected by an FDA-cleared test.

Limitations of Use:

- Patients who are CYP2D6 ultra-rapid metabolizers (URMs) may not achieve adequate concentrations of CERDELGA to achieve a therapeutic effect.
- A specific dosage cannot be recommended for those patients whose CYP2D6 genotype cannot be determined (indeterminate metabolizers).

Important Safety Information

CONTRAINDICATIONS: CERDELGA is contraindicated in the following patients based on CYP2D6 metabolizer status due to the risk of cardiac arrhythmias from prolongation of the PR, QTc, and/or QRS cardiac intervals:

- Extensive Metabolizers (EMs) taking a strong or moderate CYP2D6 inhibitor concomitantly with a strong or moderate CYP3A inhibitor, EMs with moderate or severe hepatic impairment, or EMs with mild hepatic impairment and taking a strong or moderate CYP2D6 inhibitor.
- Intermediate Metabolizers (IMs) taking a strong or moderate CYP2D6 inhibitor concomitantly with a strong or moderate CYP3A inhibitor, IMs taking a strong CYP3A inhibitor, or IMs with any degree of hepatic impairment.
- Poor Metabolizers (PMs) taking a strong CYP3A inhibitor, or PMs with any degree of hepatic impairment.

WARNINGS AND PRECAUTIONS: CERDELGA is predicted to cause increases in ECG intervals (PR, QTc, and QRS) at substantially elevated plasma concentrations and may increase risk of cardiac arrhythmias. Use of CERDELGA is contraindicated, to be avoided, or requires dosage adjustment in patients taking CYP2D6 or CYP3A inhibitors, depending CYP2D6 metabolizer status, type of inhibitor, or degree of hepatic impairment. Avoid use of CERDELGA in patients with pre-existing cardiac disease, long QT syndrome, or in combination with Class IA or Class III antiarrhythmic medications.

ADVERSE REACTIONS: The most common adverse reactions (≥10%) to CERDELGA include: fatigue, headache, nausea, diarrhea, back pain, pain in extremities, and upper abdominal pain.

DRUG INTERACTIONS: Coadministration of CERDELGA with CYP2D6 or CYP3A inhibitors may increase eliglustat concentrations, which may increase the risk of cardiac arrhythmias from prolongations of the PR, QTc, and/or QRS cardiac interval. Use of CERDELGA is contraindicated, to be avoided, or may require dosage adjustment depending on the concomitant drug and CYP2D6 metabolizer status. See section 7 of the full Prescribing Information for more details and other potentially significant drug interactions.

USE IN SPECIFIC POPULATIONS: Available data on the use of CERDELGA in pregnant women is not sufficient to assess drug-associated risks of major birth defects, miscarriage, or adverse maternal or fetal outcomes. The developmental and health benefits of breastfeeding should be considered along with the mother’s clinical need for CERDELGA and any potential adverse effects on the breastfed child from CERDELGA or from the underlying maternal condition.

Use of CERDELGA in patients with renal impairment is based on the patient’s CYP2D6 metabolizer status. Avoid use of CERDELGA in EMs with end-stage renal disease (ESRD), and IMs and PMs with any degree of renal impairment.

Use of CERDELGA is contraindicated or may require dosage adjustment in patients with hepatic impairment based on CYP2D6 metabolizer status, concomitant use of CYP2D6 or CYP3A inhibitors, and degree of hepatic impairment.

To report SUSPECTED ADVERSE REACTIONS, contact Sanofi Genzyme Medical Information at 1-800-745-4447, Option 2.

Please see accompanying full Prescribing Information.