Novartis files Exjade® New Drug Applications for treatment of chronic iron overload due to blood transfusions

- Innovative, once-a-day oral formulation offers life-altering treatment option to broad range of iron overload patients
- Filings based on data from largest prospective clinical trials program for iron chelation therapy

East Hanover, NJ, May 3, 2005 – Novartis has filed regulatory submissions for Exjade® (deferasirox), the first and only once-daily oral iron chelator for the treatment of chronic iron overload due to blood transfusions, in the United States and the European Union (EU). Submissions in other countries will follow shortly.

Exjade, also known as investigational agent ICL670, has been granted fast-track status in the US and Switzerland. Priority review has been requested in the US. Furthermore, Exjade has received Orphan Drug status in the US, EU and Australia.

An easy to administer novel oral iron chelator, Exjade is simply taken once daily, after dispersing tablets in a glass of water. Exjade was developed to extend the benefits of iron chelation to a greater number of patients receiving blood transfusions and to address the needs of thousands of adult and pediatric patients who have been using Desferal® (deferoxamine). Patients have been frustrated for years by the inconvenience and pain that can result from daily insertion of the deferoxamine infusion needle. In many patients, the need for transfusion and chelation therapy may be lifelong.

“Novartis has demonstrated a long-term commitment to help improve the lives of patients at risk for iron overload. First by developing a highly effective drug, deferoxamine, and then by conducting research on hundreds of new compounds to find an easy-to-take oral alternative to this product.” said Diane Young, MD, vice president and global head of Clinical Development at Novartis Oncology. “We understand the needs of patients and know that the burdensome administration of deferoxamine limits its use. In an effort to bring the benefits of effective iron chelation to more patients, we will work diligently with health authorities to expedite the approval of this important advancement.”
Iron overload is a life-threatening cumulative toxicity which results from lifesaving blood transfusions required to treat certain types of anemias and other disorders, including thalassemia, sickle cell disease, other rare anemias, and myelodysplastic syndromes. If left undiagnosed or untreated, iron overload can lead to damage to the liver, heart and endocrine glands. Transfused patients may require concomitant removal of excess iron with a type of drug therapy called iron chelation, to treat iron overload. Deferoxamine, the current standard of care in iron chelation, is effective but typically requires subcutaneous infusion lasting eight to twelve hours per day, for five to seven days a week for as long as the patient continues to receive blood transfusions.

“Blood transfusions can be a sickle cell disease patient’s lifeline – they reduce the occurrence of recurrent pain episodes, as well as the risk of stroke and other life-threatening complications. But with each blood transfusion, more iron can accumulate in the body until it becomes toxic and can lead to serious organ damage,” said Willarda V. Edwards, MD, president and chief operating officer, Sickle Cell Disease Association of America, Inc. “The availability of a once-daily oral iron chelator like Exjade would make it possible to eliminate excess iron without the need for the physical and emotional burden of an uncomfortable pump.”

**Filing data**

The Exjade global clinical trials program enrolled more than 1,000 patients and is the largest ever prospectively implemented for an investigational iron chelator. The filings are based on the results of pivotal clinical trials, including a Phase III head-to-head trial vs. deferoxamine, which showed that Exjade significantly reduced liver iron concentration (LIC), an accepted indicator for body iron content, in adult and pediatric patients receiving blood transfusions. Findings from the clinical trial program were presented in December 2004 at the annual meeting of the American Society of Hematology. The studies demonstrated that Exjade led to the maintenance or reduction of absolute LIC in regularly transfused patients with different underlying diseases. Additional data on Exjade will be presented this month at three important meetings: the annual meeting of the American Society of Pediatric Hematology/Oncology in Washington, D.C. (May 14-16, 2005); the 8th International Symposium on Myelodysplastic Syndromes in Nagasaki, Japan (May 12-15, 2005); and the First Congress of the International BioIron Society in Prague, Czech Republic (May 22-27, 2005); and in June at the 10th Congress of the European Hematology Association in Stockholm, Sweden (June 2-5, 2005).

In the clinical studies in both adults and children as young as two years of age, Exjade was generally well tolerated, with the most frequently reported adverse events being nausea, vomiting, diarrhea, abdominal pain, skin rash and mild stable increases in serum creatinine, usually within the normal range.

**Orphan drug designation and fast-track status**

In the EU, the filing for Exjade was submitted to the European Medicines Agency under the centralized procedure. Exjade was granted Orphan Drug status in both the US and EU in 2002. The intent of the Orphan Drug designation is to stimulate the research, development,
and approval of products that treat rare diseases. In the EU, the term “Orphan Drug” refers to a product that treats a serious or life-threatening disease that affects fewer than five people per 10,000 population. In the US, the term “Orphan Drug” refers to a product that treats a disease that affects fewer than 200,000 people in the country. Exjade also was granted fast-track status in the US and Switzerland. The fast-track designation is generally reserved for drugs intended for the treatment of a serious or life-threatening condition that demonstrate the potential to address unmet medical needs for that condition.
Additional information
Exjade is still in clinical development and not yet approved by the U.S. Food and Drug Administration (FDA). The brand name “Exjade” is also subject to approval by the FDA.
Some patients may be eligible to enroll in ongoing clinical trials. To learn more about Exjade clinical trials, patients, caregivers and their health care providers can call 800-340-6843, Monday through Friday, between 8:30 am and 5:30 pm Eastern Time.

The foregoing release contains forward-looking statements that can be identified by terminology such as “easier,” “more convenient,” “potentially,” “important advancement,” “will be,” “would represent,” “significant advance,” “would make it possible,” “fast-track designation pending,” “innovative,” “life-altering,” “lifesaving,” or similar expressions, or by express or implied discussions regarding potential additional marketing approvals or future sales of Exjade. Such forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause actual results with Exjade to be materially different from any future results, performance or achievements expressed or implied by such statements. There can be no guarantee that Exjade will receive any additional marketing approvals in any other countries, or that it will reach any particular sales levels. In particular, management's expectations regarding commercialization of Exjade could be affected by, among other things, additional analysis of Exjade clinical data; new clinical data; unexpected clinical trial results; unexpected regulatory actions or delays or government regulation generally; the company's ability to obtain or maintain patent or other proprietary intellectual property protection; competition in general; increased government, industry, and general public pricing pressures; and other risks and factors referred to in the Company's current Form 20-F on file with the U.S. Securities and Exchange Commission. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those anticipated, believed, estimated or expected. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

Full prescribing information on Desferal® (deferoxamine) is available at www.pharma.us.novartis.com/product/pi/pdf/desferal.pdf.

About Novartis
Novartis Pharmaceuticals Corporation researches, develops, manufacturers and markets leading innovative prescription drugs used to treat a number of diseases and conditions, including central nervous system disorders, organ transplantation, cardiovascular diseases, dermatological diseases, respiratory disorders, cancer and arthritis. The company's mission is to improve people's lives by pioneering novel healthcare solutions.

Located in East Hanover, New Jersey, Novartis Pharmaceuticals Corporation is an affiliate of Novartis AG (NYSE: NVS) – a world leader in pharmaceuticals and consumer health. In 2004, the Group's businesses achieved sales of USD 28.2 billion and net income of USD 5.8 billion. The Group invested approximately USD 4.2 billion in R&D.
Headquartered in Basel, Switzerland, Novartis Group companies employ approximately 81,400 people and operate in over 140 countries around the world. For further information please consult http://www.novartis.com.

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Contacts

Media Only:           Investors
Only:  

Kim Fox            Jill Pozarek
Novartis Oncology  Novartis Corporation
Tel: +1 862 778 7692
830 2445
Fax: +1 773 781 2074
kim.fox@novartis.com

Dana Kahn Cooper
Tel: +1 732 817 1800
Fax: +1 732 817 1834
Pager: 1 800 759 8888; PIN: 1062074
dkcci@juno.com

Debbie Kanterman
Tel: +1 212 715 1679
Fax: +1 212 715 1661
kantermand@ruderfinn.com