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Novartis marks Rare Disease Day with call for more research to understand and find treatments for rare diseases

- *Company is pleased to host second international conference dedicated to the research of rare and orphan diseases*
- *Novartis is engaging in public dialogue to help raise greater awareness of rare diseases and support for patients*
- *Company celebrates more than 50 years' engagement in the field of rare disease research and drug development for conditions with unmet need*

Basel, February 28, 2014 – Today Novartis joins the global rare disease community in recognition of Rare Disease Day by calling for a global exchange of ideas to deepen the understanding of rare diseases and help address a significant unmet medical need.

Rare Disease Day is an annual, international awareness-raising event coordinated globally by EURORDIS and in the US by the National Organization for Rare Disorders (NORD). The main objective of Rare Disease Day is to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives.

"Novartis is pleased to help raise the dialogue about rare diseases and the need for more research to understand these diseases better," said Dr. Mark Fishman, President of the Novartis Institutes for BioMedical Research (NIBR). "Our focus on rare diseases flows from our desire to help patients underserved by today's medicines. In addition, research into rare diseases teaches us fundamental mechanisms of human biology and disease, often applicable to more prevalent disorders."

For more than 50 years, Novartis has been a leader in the discovery and development of innovative therapies to treat rare diseases, from rare forms of cancer to debilitating genetic diseases. The focus on rare diseases began in 1963, when Ciba – one of the two predecessor companies of Novartis – launched Desferal®, a life-saving therapy for rare hemopathies. Today Novartis has nine marketed drugs that have been designated orphan drugs as well as a robust clinical pipeline including more than 40 active preclinical and clinical research projects in the rare diseases area.

While these diseases may be rare, their impact is great. There are more than 6,000 rare diseases affecting more than 60 million people across Europe and North America and millions more worldwide.[1]

For this reason, investment in both fundamental discovery and translational research is crucial. To successfully drive innovation that leads to treatments for rare diseases, Novartis believes that global efforts must continue to be focused towards understanding basic processes that go awry in such disorders, and to better understand how to translate such discoveries to new medicines. Knowledge derived from the thorough analysis of a rare disease has high scientific and societal value, because insights into

rare disorders can also provide scientists with a clear understanding of disease mechanisms that can be useful to treat more common disorders.

To this end, Novartis is pleased to sponsor and host RE(ACT) Congress 2014, the second international conference on research of rare and orphan diseases, organized by the Gebert Rüf Foundation and the Blackswan Foundation. The meeting, held on the Novartis Basel Campus from March 5th to 8th, 2014 provides the global research community with a much-needed platform to connect, exchange ideas and deepen fundamental understanding of rare diseases. For more information about RE(ACT) Congress 2014, visit http://www.react-congress.org/.

Novartis is also proud to be a global collaborator of choice for biotechnology companies and academic centers seeking to discover and develop drugs for a range of inadequately treated diseases. Novartis has established productive alliances with more than 300 collaborators, both academic and industrial, many focused on rare diseases.

“We continue to look for new ways to combine our own scientific knowledge and expertise with that of the rest of the research community, to find the shortest path to new treatments for rare diseases,” said Dr. Fishman.

Novartis is committed to finding treatments for rare diseases that improve the quality of life for patients living with a rare disease as well as to raising awareness of and supporting patients with rare diseases.

Novartis Rare Disease Day initiatives
Novartis is hosting “Rare Disease Hour” on Twitter daily from February 24th to 28th from 17:00 -18:00 CET to raise awareness about rare diseases and research efforts including messages from healthcare professionals and rare disease patients. Sign up to follow @Novartis at http://twitter.com/novartis.

A Featured Playlist with videos of interest to the rare disease community is available on the Novartis YouTube page at http://www.youtube.com/novartis.

Engaging Facebook posts to spark an ongoing dialogue on rare diseases are available at http://www.facebook.com/novartis.

An infographic detailing many of the facts and figures about rare diseases is available at www.novartis.com/stories/medicines/2014-02-rare-disease-infographic.shtml.

Learn more about why Novartis researchers are so passionate about finding treatments for rare diseases at www.novartis.com/stories/medicines/2014-02-rare-disease-research.shtml.

Novartis is proud to sponsor and host RE(ACT) Congress 2014, the second international conference on research of rare and orphan diseases, organized by the Gebert Rüf Foundation and the Blackswan Foundation, on the Novartis Basel, Switzerland campus from March 5th to 8th, 2014. This international congress brings together scientists from different disciplines – including stem cell researchers, geneticists, biochemists, clinicians and pharmacists – to discuss rare diseases and the quest for potential treatments.

Novartis research commitment to rare diseases
Novartis is leading in researching and developing innovative therapies to help address the high unmet medical need in rare diseases. Rare diseases are a key strategic focus of the research strategy at NIBR where scientists are currently investigating treatments for more than 40 rare diseases. These research programs have more than 20 active orphan drug designations from the US Food and Drug Administration and European Medicines Agency or both, targeting disorders ranging from aggressive systemic mastocytosis and

Cushing's disease to sporadic Inclusion Body Myositis (sIBM) and spinal muscular atrophy.

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About Novartis
Novartis provides innovative healthcare solutions that address the evolving needs of patients and societies. Headquartered in Basel, Switzerland, Novartis offers a diversified portfolio to best meet these needs: innovative medicines, eye care, cost-saving generic pharmaceuticals, preventive vaccines and diagnostic tools, over-the-counter and animal health products. Novartis is the only global company with leading positions in these areas. In 2013, the Group achieved net sales of USD 57.9 billion, while R&D throughout the Group amounted to approximately USD 9.9 billion (USD 9.6 billion excluding impairment and amortization charges). Novartis Group companies employ approximately 136,000 full-time-equivalent associates and operate in more than 140 countries around the world.
For more information, please visit http://www.novartis.com.

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