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Video: New Biological Therapy Ilaris® Approved in US to Treat Children and Adults with CAPS, a Serious Life-Long Auto-Inflammatory Disease

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- Phase III data show rapid and sustained remission in more than 90% of Ilaris-treated patients with cryopyrin-associated periodic syndrome (CAPS)

- Ilaris is the first approved treatment for children as young as four and is given only every eight weeks

- Highly selective in targeting and blocking interleukin-1 beta (IL-1 beta), which is overproduced in CAPS patients causing inflammation

- Studies under way in other inflammatory disorders involving IL-1 beta, such as some forms of gout, systemic juvenile idiopathic arthritis and type 2 diabetes

EAST HANOVER, N.J., June 18 /PRNewswire/ -- The U.S. Food and Drug Administration (FDA) has approved Ilaris® (canakinumab) for the treatment of children and adults with cryopyrin-associated periodic syndrome (CAPS), which includes a number of rare, but life-long, auto-inflammatory disorders with debilitating symptoms and limited treatment options. The FDA granted priority review to Ilaris based on its potential to meet an important clinical need for patients with CAPS.

To view the Multimedia News Release, go to: http://www.prnewswire.com/mnr/novartis/38814/

Ilaris is the first approved treatment for patients as young as four years old suffering from two forms of CAPS: familial cold auto-inflammatory syndrome (FCAS) and Muckle-Wells syndrome (MWS).

CAPS is caused by a single gene mutation that leads to overproduction of interleukin-1 beta (IL-1 beta), which causes sustained inflammation and tissue damage. Symptoms, such as debilitating fatigue, rash, fever, headaches, joint pain and conjunctivitis, can be present from birth or infancy, and occur daily throughout patients' lives. Long-term consequences may be serious and potentially fatal, and include deafness and amyloidosis resulting in renal failure and early death.

Ilaris, previously known as ACZ885, is a fully human monoclonal antibody that rapidly and selectively blocks interleukin-1 beta (IL-1 beta). The dosing schedule for Ilaris is once every eight weeks, which is less frequent than the current approved therapy. Also, more than 90% of patients studied did not experience any injection site reactions and among those that did these were of a mild-to-moderate nature.

"Until now, treatments for CAPS patients have been limited to traditional inflammatory-disease medications that work by suppressing the entire immune system, and newer therapies that control the disease better but require more frequent injections," said Hal Hoffman, MD, Associate Professor of Pediatrics Medicine at University of California, San Diego, USA.

He added, "CAPS is a life-long disease and the convenience of administration of medicine is of the utmost importance for these patients. With rapid and sustained response, good tolerability, and a significantly less-frequent dosing schedule, Ilaris represents an important treatment advance for children and adults with CAPS."

CAPS comprises three disorders of increasing severity: FCAS, MWS and neonatal-onset multisystem inflammatory disease (NOMID). There are believed to be approximately 300 cases in the US, but many patients may remain undiagnosed due to poor disease recognition. A clinical study is ongoing to evaluate the potential of Ilaris to treat patients with NOMID. There are currently no approved therapies for the treatment of NOMID.

"Children and adults affected by these inflammatory diseases have to cope daily with distressing and debilitating symptoms," said Trevor Mundel, MD, Head of Global Development at Novartis Pharma AG. "We are excited about this first Ilaris approval which reflects our commitment to developing innovative treatments that address unmet medical needs, regardless of the size of the patient population."

In addition to ongoing studies in CAPS, clinical trials are also under way with Ilaris in systemic juvenile idiopathic arthritis (SJIA), and more common disorders such as some forms of gout, chronic obstructive pulmonary disorder (COPD) and type 2 diabetes. Further development in rheumatoid arthritis (RA) is not planned.

The approval of Ilaris is based on a three-part, one-year Phase III study involving 35 patients aged nine to 74 years old with varying degrees of disease severity. Results published in The New England Journal of Medicine on June 4, 2009 show that Ilaris produced a rapid, complete and sustained response in the majority of patients.

Part two of the study included the primary endpoint, a comparison between the number of patients treated

every two months with Ilaris who experienced disease outbreaks or 'flares' vs. those on placebo. Results showed that none of the patients in the Ilaris group (0 out of 15) experienced a disease flare compared to 13 out of 16 patients in the placebo group (0% vs. 81%, respectively, $p<0.001$).

Ilaris was generally well tolerated, with no consistent pattern of adverse events beyond an increase in all suspected infections. Two patients experienced serious adverse events, which were a lower urinary tract infection and vertigo. The most common adverse events reported by patients treated with Ilaris were nasopharyngitis, diarrhea, influenza, headache and nausea. No impact on the type or frequency of adverse events was seen with longer-term treatment.

Ilaris has orphan drug designation for CAPS in the US, as well as in the EU, Switzerland, and Australia, where it is currently under health authority review. Priority review has been granted in Switzerland, Australia and Canada. Ilaris has been granted orphan drug designation for SJIA in the US, EU and Switzerland, and also has fast-track status for SJIA in the US. Orphan drugs are those developed to treat diseases affecting fewer than 200,000 people (in the US) or fewer than five out of 10,000 people (in the EU).

Important Safety Information

Ilaris may affect the immune system and may lower the ability to fight infections. Infections, in some instances serious, have been reported after treatment. Ilaris should be used with caution in patients with an infection and discontinued if a serious infection develops. Patients should receive all recommended vaccinations prior to initiation of treatment and live vaccines should not be used in treated patients. Vertigo has been reported in patients treated with Ilaris. The most common side effects are inflammation of the upper airways, diarrhea, flu-like symptoms, headache and nausea. There are no studies in pregnant women with Ilaris and it should be used during pregnancy only if clearly needed.

For full Ilaris prescribing information, go to www.pharma.us.novartis.com.

Disclaimer

The foregoing release contains forward-looking statements that can be identified by terminology such as "priority review," "potential," "can," "may," "potentially," "believed," "commitment," "planned," "fast-track status," or similar expressions, or by express or implied discussions regarding potential future regulatory filings or marketing approvals for Ilaris or regarding potential future revenues from Ilaris. You should not place undue reliance on these statements. Such forward-looking statements reflect the current views of the Company regarding future events, and involve known and unknown risks, uncertainties and other factors that may cause actual results with Ilaris to be materially different from any future results, performance or achievements expressed or implied by such statements. There can be no guarantee that Ilaris will be approved for sale in any additional market, or for any additional indication. Nor can there be any guarantee that Ilaris will achieve any levels of revenue in the future. In particular, management's expectations regarding Ilaris could be affected by, among other things, unexpected clinical trial results, including unexpected new clinical data and unexpected additional analysis of existing clinical data; 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; government, industry and general public pricing pressures; the impact that

the foregoing factors could have on the values attributed to the Group's assets and liabilities as recorded in the Group's consolidated balance sheet, and other risks and factors referred to in Novartis AG's current Form 20-F on file with the US 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.

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