Novartis announces FDA filing acceptance of Xolair® (omalizumab) prefilled syringe for self-administration across all indications

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- If approved, Xolair self-administration would offer a more flexible option to help select patients manage their treatment needs
- Filing acceptance is based on the well-established efficacy and safety profile of Xolair in allergic asthma and chronic idiopathic urticaria

EAST HANOVER, N.J., Aug. 13, 2020 /PRNewswire/ -- Novartis today announced that the US Food and Drug Administration (FDA) accepted the company's supplemental Biologics License Application (sBLA) for a new self-administration option for Xolair® (omalizumab) across all approved US indications. If approved, Xolair prefilled syringe would become available for either self-administration by select patients or administration by their caregivers. A decision on approval is anticipated by Q1 2021. In the US, Xolair is currently approved for administration by a healthcare provider in a healthcare setting, and is the only approved biologic designed to target and block immunoglobulin E (IgE) for the treatment of patients with moderate to severe persistent allergic asthma and chronic idiopathic urticaria (CIU).

"Today's FDA filing acceptance of Xolair is a big step forward for people living with IgE-mediated conditions," said Victor Bultó, President, Novartis Pharmaceuticals Corporation. "If approved, self-administration of Xolair would offer a new, convenient and flexible treatment administration option for patients and healthcare providers, which builds on the proven legacy, safety profile and efficacy of Xolair over the last 17 years of real-world use. This is in line with Novartis' strong heritage of innovation and ongoing commitment to reimagining medicine and understanding the continually changing needs of patients and healthcare providers."

If approved, once Xolair therapy has been established and closely observed by a healthcare provider, self-administration of Xolair prefilled syringe outside of a healthcare setting by a patient or caregiver may be deemed appropriate by the healthcare provider for select patients. In those instances, the patient or caregiver would be trained by a healthcare provider in the correct subcutaneous injection technique and recognition of the early signs and symptoms of anaphylaxis.

Approximately 460,000 patients have been treated in the US with Xolair since its initial approval for allergic asthma in 2003.[1] The use of Xolair in allergic asthma and CIU is supported by a robust clinical development program, including eight Phase III studies. Independent clinical studies involving patients with allergic asthma and CIU on Xolair treatment suggest that Xolair may be self-administered with proper training and monitoring.[2] In the US, Novartis Pharmaceuticals Corporation and Genentech work together to develop and co-promote Xolair.

Xolair is the only IgE blocking biologic available for the treatment of moderate to severe persistent allergic asthma in people six years of age or older whose asthma symptoms are not controlled by inhaled corticosteroids, and for CIU in people 12 years of age and older who continue to have hives that are not controlled by H1 antihistamines.

The FDA filing acceptance follows the European Commission's approval for Xolair self-administration (or administration by a trained caregiver) for approved indications in December 2018.

About Allergic Asthma and Chronic Idiopathic Urticaria

Asthma is a serious and chronic lung disease affecting an estimated 24 million people in the US.[3,4] It causes swelling and narrowing of the airways, making breathing difficult. Allergic asthma, the most common form of asthma, accounts for approximately 60 percent of asthma cases in adults.[5,6]

Chronic idiopathic urticaria (CIU) is a skin condition that can cause hives and severe itch that can last many months and years.[7] CIU is characterized by hives that spontaneously occur without an identifiable cause, and recur for six weeks or more.[8] It is estimated that approximately 1.5 million people in the US have CIU.[7,9]

About Xolair® (omalizumab)

Xolair (omalizumab) is the only approved antibody designed to target and block immunoglobulin E (IgE). By reducing free IgE, down-regulating high-affinity IgE receptors and limiting mast cell degranulation, Xolair minimizes the release of mediators throughout the allergic inflammatory cascade.

An injectable prescription medicine, Xolair is approved for the treatment of moderate-to-severe or severe persistent allergic asthma in more than 90 countries, including the US since 2003 and the EU since 2005. Xolair is approved for the treatment of chronic spontaneous urticaria in over 80 countries including the European Union and for chronic idiopathic urticaria (CIU), as it is known in the US and Canada. Xolair has over one million patient years of exposure. In addition, a liquid formulation of Xolair in prefilled syringes has been approved in the US, EU and more than 10 countries outside of the EU, including Canada and Australia. The self-administration indication for Xolair in prefilled syringes was also approved in the EU in 2018. Outside the US, Novartis markets Xolair and records all sales and related costs.

Xolair US Indications

Xolair for subcutaneous use is an injectable prescription medicine approved by the FDA to treat:

- Moderate to severe persistent asthma in patients six years of age or older whose asthma symptoms are not controlled by asthma medicines called inhaled corticosteroids. A skin or blood test is performed to see if a patient has allergies to year-round allergens.
- Chronic idiopathic urticaria (CIU; chronic hives without a known cause) in patients 12 years of age and older who continue to have hives that are not controlled by H1 antihistamine treatment.

Xolair is not used to treat other allergic conditions, other forms of urticaria, acute bronchospasm or status asthmaticus.

Important Safety Information

The most important information patients should know about XOLAIR is that a severe allergic reaction called anaphylaxis can happen when you receive XOLAIR. The reaction can occur after the first dose, or after many doses. It may also occur right after a XOLAIR injection or days later. Anaphylaxis is a life-threatening condition and can lead to death. Go to the nearest emergency room right away if you have any of these symptoms of an allergic reaction:

- wheezing, shortness of breath, cough, chest tightness, or trouble breathing
- low blood pressure, dizziness, fainting, rapid or weak heartbeat, anxiety, or feeling of "impending doom"
- flushing, itching, hives, or feeling warm

- swelling of the throat or tongue, throat tightness, hoarse voice, or trouble swallowing

The patient's healthcare provider will monitor you closely for symptoms of an allergic reaction while you are receiving XOLAIR and for a period of time after the patient's injection. The patient's healthcare provider should talk to the patient about getting medical treatment if they have symptoms of an allergic reaction after leaving the healthcare provider's office or treatment center.

Patients must not receive XOLAIR if they are allergic to omalizumab or any of the ingredients.

Before receiving XOLAIR, patients must tell their healthcare provider about all of their medical conditions, including if they:

- have a latex allergy or any other allergies (such as food allergy or seasonal allergies). The needle cap on the XOLAIR prefilled syringe may contain latex
- have sudden breathing problems (bronchospasm)
- have ever had a severe allergic reaction called anaphylaxis
- have or have had a parasitic infection
- have or have had cancer
- are pregnant or plan to become pregnant. It is not known if XOLAIR may harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if XOLAIR passes into your breast milk. Talk with your healthcare provider about the best way to feed your baby while you receive XOLAIR.

Patients must tell their healthcare provider about all the medicines they take, including prescription and over-the-counter medicines, vitamins, or herbal supplements.

Receiving XOLAIR

- XOLAIR should be given by your healthcare provider, in a healthcare setting.
- XOLAIR is given in 1 or more injections under the skin (subcutaneous), 1 time every 2 or 4 weeks.
- In asthma patients, a blood test for a substance called IgE must be performed prior to starting XOLAIR to determine the appropriate dose and dosing frequency.
- In patients with chronic hives, a blood test is not necessary to determine the dose or dosing frequency.
- Do not decrease or stop taking any of your other asthma or hive medicine unless your healthcare providers tell you to.
- You may not see improvement in your symptoms right away after XOLAIR treatment.

Possible Side Effects of XOLAIR

XOLAIR may cause serious side effects, including:

- See, "**What is the most important information I should know about XOLAIR**" in the XOLAIR Medication Guide at http://www.xolair.com regarding the risk of anaphylaxis.
- **Cancer**. Cases of cancer were observed in some people who received XOLAIR.
- **Inflammation of your blood vessels**. Rarely, this can happen in people with asthma who receive XOLAIR. This usually, but not always, happens in people who also take a steroid medicine by mouth that is being stopped or the dose is being lowered. It is not known whether this is caused by XOLAIR. Tell your healthcare provider right away if you have rash; chest pain; shortness of breath; or a feeling of pins and needles or numbness of your arms or legs.
- **Fever, muscle aches, and rash**. Some people who take XOLAIR get these symptoms 1 to 5 days after receiving a XOLAIR injection. If you have any of these symptoms, tell your healthcare provider.
- **Parasitic infection**. Some people who are at a high risk for parasite (worm) infections, get a parasite

infection after receiving XOLAIR. Your healthcare provider can test your stool to check if you have a parasite infection.
- **Heart and circulation problems**. Some people who receive XOLAIR have had chest pain, heart attack, blood clots in the lungs or legs, or temporary symptoms of weakness on one side of the body, slurred speech, or altered vision. It is not known whether this is caused by XOLAIR.

The most common side effects of XOLAIR:

- **In adults and children 12 years of age and older with asthma:** pain especially in your arms and legs, dizziness, feeling tired, skin rash, bone fractures, and pain or discomfort of your ears.
- **In children 6 to less than 12 years of age with asthma:** common cold symptoms, headache, fever, sore throat, pain or discomfort of your ear, abdominal pain, nausea, vomiting and nose bleeds.
- **In people with chronic idiopathic urticaria:** nausea, headaches, swelling of the inside of your nose, throat or sinuses, cough, joint pain, and upper respiratory tract infection.

These are not all the possible side effects of XOLAIR. Patients should call their doctor for medical advice about side effects.

Report side effects to the FDA at (800) FDA-1088 or http://www.fda.gov/medwatch**. Report side effects to Genentech at (888) 835-2555 or Novartis Pharmaceuticals Corporation at (888) 669-6682.**

Please see full Prescribing Information, including Medication Guide at http://www.xolair.com **for additional Important Safety Information.**

About Novartis
Located in East Hanover, NJ Novartis Pharmaceuticals Corporation – an affiliate of Novartis – is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis employs about 15,000 people in the United States. For more information, please visit **https://www.novartis.us.**

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Novartis Media Relations

E-mail: media.relations@novartis.com

Jamie Bennett	**Martin DeBenedetto**
Director, US Media Relations	Director, Communications
+1 862 217 3976	(862) 778 7619 (direct)
jamie.bennett@novartis.com	(973) 738 4104 (mobile)
	martin.debenedetto@novartis.com

Novartis Investor Relations

E-mail: investor.relations@novartis.com

Sloan Simpson +1 862 778 5052

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