FDA approves Xolair® (omalizumab) as first and only medicine for children and adults with one or more food allergies

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- Approval is based on data from the NIH-sponsored Phase III OUtMATCH study, which showed a significantly higher proportion of food allergy patients as young as 1 year treated with Xolair could tolerate small amounts of peanut, milk, egg and cashew without an allergic reaction, compared to placebo
- More than 40% of children and more than half of adults with food allergies have experienced a severe reaction at least once[1,2]
- Detailed OUtMATCH results will be featured in a late-breaking symposium at the 2024 AAAAI Annual Meeting

EAST HANOVER, N.J., Feb. 16, 2024 -- Novartis announced today that the US Food and Drug Administration (FDA) has approved Xolair® (omalizumab) for the reduction of allergic reactions, including anaphylaxis, that may occur with accidental exposure to one or more foods in adult and pediatric patients aged 1 year and older with IgE-mediated food allergy. People taking Xolair for food allergies should continue to avoid all foods they are allergic to (commonly referred to as "food allergen avoidance"). Xolair should not be used for the emergency treatment of any allergic reactions, including anaphylaxis. Immunoglobulin E (IgE)-mediated food allergies are the most common type and are typically characterized by the rapid onset of symptoms following exposure to certain food allergens[3]. Xolair is the first and only FDA-approved medicine to reduce allergic reactions in people with one or more food allergies. Xolair is widely available and can now be prescribed for appropriate patients with IgE-mediated food allergy in the US.

"Many people with food allergies and their loved ones live in constant fear of accidentally coming into contact with the food they are allergic to and the life-threatening allergic reaction that could happen as a result. Today's approval of Xolair represents a paradigm shift in the way food allergies can be managed," said Reshema Kemps-Polanco, Executive Vice President and Chief Commercial Officer, Novartis US. "This approval, which comes 20 years after the first approval for this medicine, shows that we never stop innovating to make a meaningful difference for patients living with immunological conditions."

The FDA approval is based on positive data from the Phase III OUtMATCH study, which evaluated Xolair in patients aged 1 to 55 years allergic to peanuts and at least two other food allergens, including milk, egg, wheat, cashew, hazelnut and walnut. The OUtMATCH study is sponsored and funded by the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health (NIH), and is being conducted by the NIAID-funded Consortium for Food Allergy Research (CoFAR) at 10 clinical sites across the US led by Johns Hopkins Children's Center and co-led by Stanford School of Medicine. Detailed results from the study will be featured in a late-breaking symposium at the 2024 American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting on Sunday, February 25.

"As more and more people are affected by food allergies, the need for a new approach to help prevent serious and often life-threatening allergic reactions and emergencies is critical," said Sung Poblete, R.N., Ph.D., CEO of FARE (Food Allergy Research and Education). "As someone with food allergies, I know firsthand the significant impact they can have on people and their loved ones, and I share in the community's excitement for this approval."

"The stress of living with food allergies can weigh heavily on people and their families, particularly when navigating events like children's birthday parties, school lunches, and holiday dinners with friends and family," said Kenneth Mendez, President and CEO of the Asthma and Allergy Foundation of America (AAFA). "Given the growing prevalence of food allergies, this news offers hope to the many children and adults who may benefit from a new way to help manage their food allergies."

Patients entered the OUtMATCH study unable to tolerate up to 100 mg of peanut protein (equivalent to about one third of a peanut), and up to 300 mg each of milk, egg and cashew protein. After 16 to 20 weeks of treatment with Xolair or placebo, each participant completed four food challenges, receiving gradually increasing amounts of foods they are allergic to (and a placebo ingredient), in order to assess their ability to consume a single dose of at least 600 mg of peanut protein (primary endpoint), and a single dose of at least 1,000 mg of milk, egg or cashew protein (secondary endpoints) without experiencing moderate to severe allergic symptoms.

Study results showed a statistically significant higher proportion of patients (68%) treated with Xolair for 16 to 20 weeks tolerated at least 600 mg of peanut protein without moderate to severe allergic symptoms, compared to 5% of those treated with placebo ($p<0.0001$). This amount is equivalent to approximately two and a half peanuts or half a teaspoon of regular peanut butter.

In addition, a statistically significant higher proportion of patients treated with Xolair compared to placebo tolerated at least 1,000 mg of protein from milk (66% vs. 11%; $p<0.0001$), egg (67% vs. 0%; $p<0.0001$) or cashew (42% vs. 3%; $p<0.0001$) without moderate to severe allergic symptoms. This amount is equivalent to approximately two tablespoons of 1% milk, one-quarter of an egg or three and a half cashews. While patients in the study tolerated these amounts of food, treatment with Xolair should be used with continued food allergen avoidance.

Safety findings were consistent with the known safety profile of Xolair across its additional indications and in previous clinical trials[4]. The most common adverse events (≥3% of patients) in Xolair-treated patients in the study were injection site reaction (15.5% vs. 10.9% with placebo) and fever (6.4% vs. 3.6% with placebo).

About 3.4 million children and 13.6 million adults in the US have been diagnosed with IgE-mediated food allergies, based on estimates for 2024[1,2]. Food allergy prevalence has been on the rise for the past 20 years[5]. There are 160 different foods that cause IgE-mediated food allergy[6]. Allergic reactions can range from mild to moderate, including hives and swelling, to severe and life-threatening, such as anaphylaxis. More than 40% of children and more than half of adults with food allergies have experienced a severe reaction at least once, and it is estimated that food-related anaphylaxis results in 30,000 medical events treated in emergency rooms in the US each year[1,2,7].

This marks the fourth FDA-approved indication for Xolair across allergic and inflammatory conditions, including moderate to severe persistent allergic asthma, chronic spontaneous urticaria (CSU) and chronic rhinosinusitis with nasal polyps (CRSwNP). Since its initial approval in 2003, more than 700,000 patients have been treated with Xolair in the US[8].

Xolair is a prescription biologic medicine that is given as an injection under the skin (subcutaneous). It is the only FDA-approved antibody designed to target and block IgE — an underlying driver of food allergy reactions. The recommended Xolair dosage for treatment of food allergy is 75 mg to 600 mg once every 2 or 4 weeks. Xolair dose and dosing frequency is determined by total serum IgE level and body weight. Injections can be given by a healthcare provider in a healthcare setting or at home through self-injection after initiating in a healthcare setting. Healthcare providers will determine appropriate candidates for self-injection.

Genentech and Novartis are committed to helping people access the medicines they are prescribed and offer comprehensive services for people prescribed Xolair to help minimize barriers to access and reimbursement. More information is available at 866-4ACCESS/866-422-2377.

In the US, Novartis Pharmaceuticals Corporation and Genentech, a member of the Roche Group, work together to develop and co-promote Xolair.

About the OUtMATCH Study
The Omalizumab as Monotherapy and as Adjunct Therapy to Multi-Allergen Oral Immunotherapy in Food Allergic Children and Adults (OUtMATCH; NCT03881696) study is a three-stage, multicenter, randomized, double-blind, placebo-controlled study evaluating Xolair safety and efficacy in patients aged 1 to 55 years who are allergic to peanuts and at least two other food allergens.

Stage 1 patients were randomized to receive placebo or Xolair injections either every two weeks or every four weeks for 16 to 20 weeks. The Xolair dose and dosing interval were determined by total serum immunoglobulin E (IgE) level and body weight at baseline.

After 16 to 20 weeks of treatment with Xolair or placebo, each participant completed four separate blinded food challenges where they were given gradually increasing amounts of peanut protein, two other food proteins they were allergic to, and a placebo ingredient. The food challenges were conducted in a carefully controlled setting with investigators looking for signs and symptoms of allergic reaction to assess patients' ability to consume a single dose of at least 600 mg of peanut protein (primary endpoint), and a single dose of at least 1,000 mg of milk, egg, wheat, cashew, hazelnut or walnut protein (secondary endpoints) without experiencing dose-limiting symptoms, which were defined as moderate to severe allergic symptoms, including skin, respiratory or gastrointestinal symptoms.

The OUtMATCH study is sponsored and funded by the National Institute of Allergy and Infectious Diseases (NIAID), part of the NIH, and is being conducted by the NIAID-funded Consortium for Food Allergy Research (CoFAR) at 10 clinical sites across the US led by Johns Hopkins Children's Center and co-led by Stanford School of Medicine. The study is also supported by Genentech and Novartis Pharmaceuticals Corporation.

About Xolair
In the US, Xolair 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.

Indications and Important Safety Information
What is XOLAIR?

XOLAIR® (omalizumab) for subcutaneous use is an injectable prescription medicine used to treat:

- moderate to severe persistent asthma in people 6 years of age and older whose asthma symptoms are not well controlled with asthma medicines called inhaled corticosteroids. A skin or blood test is performed to see if you have allergies to year-round allergens. It is not known if XOLAIR is safe and effective in people with asthma under 6 years of age.

- chronic rhinosinusitis with nasal polyps (CRSwNP) in people 18 years of age and older when medicines to treat CRSwNP called nasal corticosteroids have not worked well enough. It is not known if XOLAIR is safe and effective in people with CRSwNP under 18 years of age.

- food allergy in people 1 year of age and older to reduce allergic reactions that may occur after accidentally eating one or more foods to which you are allergic. While taking XOLAIR you should continue to avoid all foods to which you are allergic. It is not known if XOLAIR is safe and effective in people with food allergy under 1 year of age.

- chronic spontaneous urticaria (CSU, previously referred to as chronic idiopathic urticaria (CIU), chronic hives

without a known cause) in people 12 years of age and older who continue to have hives that are not controlled with H1 antihistamine treatment. It is not known if XOLAIR is safe and effective in people with CSU under 12 years of age.

XOLAIR should not be used for the emergency treatment of any allergic reactions, including anaphylaxis. XOLAIR should also not be used to treat other forms of hives, or sudden breathing problems.

IMPORTANT SAFETY INFORMATION

What is the most important information I should know about XOLAIR?
Severe allergic reaction. 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

Your healthcare provider will monitor you closely for symptoms of an allergic reaction while you are receiving XOLAIR and for a period of time after treatment is initiated. Your healthcare provider should talk to you about getting medical treatment if you have symptoms of an allergic reaction.

Do not receive and use XOLAIR if you are allergic to omalizumab or any of the ingredients in XOLAIR.

Before receiving XOLAIR, tell your healthcare provider about all of your medical conditions, including if you:

- have a latex allergy or any other allergies (such as seasonal allergies). The needle cap on the XOLAIR prefilled syringe contains a type of natural rubber 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 and use XOLAIR.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

How should I receive and use XOLAIR?

- When starting treatment XOLAIR should be given by your healthcare provider in a healthcare setting.
- If your healthcare provider decides that you or a caregiver may be able to give your own XOLAIR prefilled syringe or autoinjector injections, you should receive training on the right way to prepare and inject XOLAIR.
- Do not try to inject XOLAIR until you have been shown the right way to give XOLAIR prefilled syringe or autoinjector injections by a healthcare provider. Use XOLAIR exactly as prescribed by your healthcare provider.
- The XOLAIR autoinjector (all doses) is intended for use only in adults and adolescents aged 12 years and older. For children 12 years of age and older, XOLAIR prefilled syringe or autoinjector may be self-injected under adult supervision. For children 1 to 11 years of age, XOLAIR prefilled syringe should be injected by a caregiver.

- See the detailed Instructions for Use that comes with XOLAIR for information on the right way to prepare and inject XOLAIR.
- XOLAIR is given in 1 or more injections under the skin (subcutaneous), 1 time every 2 or 4 weeks.
- In people with asthma, CRSwNP and food allergy, a blood test for a substance called IgE must be performed before starting XOLAIR to determine the appropriate dose and dosing frequency.
- In people 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, CRSwNP, hive medicine, food allergy medicine or allergen immunotherapy, unless your healthcare providers tell you to.
- You may not see improvement in your symptoms right away after XOLAIR treatment. If your symptoms do not improve or get worse, call your healthcare provider.
- If you inject more XOLAIR than prescribed, call your healthcare provider right away.

What are the possible side effects of XOLAIR?

XOLAIR may cause serious side effects, including:

- 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 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 these are caused by XOLAIR.

The most common side effects of XOLAIR:

- In adults and children 12 years of age and older with asthma: joint pain especially in your arms and legs, dizziness, feeling tired, itching, skin rash, bone fractures, and pain or discomfort of your ears.
- In children 6 to less than 12 years of age with asthma: swelling of the inside of your nose, throat, or sinuses, headache, fever, throat infection, ear infection, abdominal pain, stomach infection, and nose bleeds.
- In adults with chronic rhinosinusitis with nasal polyps: headache, injection site reactions, joint pain, upper abdominal pain, and dizziness.
- In people with chronic spontaneous urticaria: nausea, headaches, swelling of the inside of your nose, throat or sinuses, cough, joint pain, and upper respiratory tract infection.
- In people with food allergy: injection site reactions and fever.

These are not all the possible side effects of XOLAIR. Call your doctor for medical advice about side effects.

You may report side effects to the FDA at (800) FDA-1088 or www.fda.gov/medwatch. You may also 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 for additional Important Safety Information and Instructions for Use.

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About Novartis

Novartis is a focused innovative medicines company. Every day, we work to reimagine medicine to improve and extend people's lives so that patients, healthcare professionals and societies are empowered in the face of serious disease. Our medicines reach more than 250 million people worldwide.

Reimagine medicine with us: Visit us at https://www.novartis.com and https://www.novartis.us and connect with us on LinkedIn, LinkedIn US, Facebook, X/Twitter, X/Twitter US and Instagram.

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