LFA answers to Frequently Asked Questions about BENLYSTA®

Updated: March 16, 2011 -

1. What is BENLYSTA?

BENLYSTA is a human monoclonal antibody that was approved for the treatment of lupus by the U.S. Food and Drug Administration (FDA) on March 9, 2011. A monoclonal antibody is a type of protein made in the laboratory that is developed to find and attach to only one type of substance in the body.

2. How does BENLYSTA work?

BENLYSTA is a human monoclonal antibody that specifically recognizes and blocks the biological activity of B-lymphocyte stimulator, or BLyS® (pronounced bliss), a naturally occurring protein which was discovered by scientists at Human Genome Sciences (HGS). Elevated levels of BLyS prolong the survival of B cells which can contribute to the production of autoantibodies – antibodies that target the body's own tissues. Studies have shown that BENLYSTA can reduce autoantibody levels and help control autoimmune disease activity.

3. Who developed BENLYSTA?

BENLYSTA was co-developed by Human Genome Sciences (HGS) and GlaxoSmithKline (GSK).

4. What does an FDA approval of BENLYSTA mean for people with lupus?

BENLYSTA represents a breakthrough in the treatment of lupus. BENLYSTA is the **first drug approved to treat lupus in more than 50 years AND is the first drug developed specifically for lupus** since the disease was discovered **more than a century ago**! Successful treatment of lupus will require an arsenal of safe, effective, and tolerable treatments. The approval of BENLYSTA is a significant first step toward reaching that goal.

5. What makes BENLYSTA different from other lupus treatments?

BENLYSTA is the **FIRST FDA-approved medication specifically designed for the treatment of lupus**. BENLYSTA targets specific immune cells, rather than the blanket approach of other therapies which suppress the entire immune system. Currently approved medications for lupus are borrowed from other diseases and conditions; other treatments are used off-label, which means they were never approved by the FDA for lupus. Many of these treatments have serious and devastating side effects.

These drugs include high doses of steroids, antimalarial medications, immunosupressive drugs, and organ-rejection drugs.

6. When will BENLYSTA be available for patients?

According to Human Genome Sciences, BENLYSTA will be available to physicians and patients before the end of March 2011. It is best to talk to your doctor about the availability of BENLYSTA and if it is right for you.

7. Where can I find out more information about BENLYSTA?

BENLYSTA Gateway is a centralized resource for patients and health care providers for one-on-one services and support. For more information call 1-877-4-BENLYSTA, Monday through Friday, 8AM to 8PM (EST). You can also visit www.benlysta.com for more information, or benlystahcp.com.

8. Who should take BENLYSTA? Will it work for everyone?

Each person with lupus is unique, and BENLYSTA will not be an option for everyone. You will need to discuss with your doctor if BENLYSTA may be an appropriate treatment option for you.

BENLYSTA is approved for the treatment of adult patients with active, autoantibody-positive systemic lupus erythematosus (SLE) who are receiving standard therapy.

The label for BENLYSTA includes the following limitations of use: The efficacy of BENLYSTA has not been evaluated in patients with severe active lupus nephritis or severe active central nervous system lupus, and has not been studied in combination with other biologics or intravenous cyclophosphamide. Use of BENLYSTA is therefore not recommended in these situations.

9. Is BENLYSTA effective?
Lupus is a complex disease, and BENLYSTA is not be right for everyone. Lupus patients are on strong background medications which impact the margin of effectiveness between the placebo and treatment group. In two major clinical trials, BENLYSTA achieved statistical significance over these medications. These are also the first lupus trials to meet its primary endpoints.

10. Is BENYLSTA effective for African-Americans?
The population size of African Americans in the study was not large enough to determine the effectiveness of BENLYSTA in that population. Additional research is needed. Human Genome Sciences and GlaxoSmithKline have already committed to doing a follow-up study that will evaluate the effectiveness of BENLYSTA in African Americans. We encourage you to visit the LFA's Center for Clinical Trials Education (www.lupus.org/clinicaltrials) to learn more about future trials in your community through the Lupus Research Registry.

11. Is BENLSYTA approved for use in children with lupus?
No. Additional studies are required before BENLYSTA can be approved for use in children.

12. Will there be any further clinical trials on BENLYSTA?
Yes, additional trials are being planned. To learn more about clinical trials, visit lupus.org/clinicaltrials. On the Website you can also join our Lupus Research Registry to stay informed about lupus trials in your community.

13. Is BENLYSTA effective for individuals with lupus who have organ involvement?
BENLYSTA was not studied for use in individuals with severe lupus nephritis, or active central nervous system involvement. BENLYSTA is not currently recommended for those with CNS or kidney involvement.

14. What side effects have been found with BENLYSTA?
BENLYSTA was generally well-tolerated. The most commonly reported adverse reactions with BENLYSTA were nausea, diarrhea, fever, inflammation of the nose and throat, bronchitis, insomnia, pain in extremity, depression, and migraine.

15. How is BENLYSTA administered?
BENLYSTA is administered by a medical professional through an IV (intravenous) infusion directly into the vein.

16. Should I receive vaccinations if I am taking BENLYSTA?
Overall, vaccinations are considered to be safe and effective for people with lupus, However, it is not recommended for people with lupus to receive vaccines with a form of attenuated (weakened, but still live) virus and should not be exposed to recent recipients of live-vaccines such as oral polio virus (OPV). It is recommended that individuals always speak with their physician prior to receiving a vaccine.

17. How much does BENLYSTA cost?
The cost of BENLYSTA is in line with similar therapies. It will be important for insurance companies to cover BENLYSTA since it is the first and only treatment developed specifically for lupus. The LFA will be working to ensure that insurance companies cover BENLYSTA on their formularies. We also encourage you to inquire about financial assistance programs at your local hospital. Human Genome Sciences and GlaxoSmithKline will also be offering a Patient Assistance Program. For additional information and questions about eligibility call BENLYSTA Gateway 1-877-4-BENLYSTA, or visit www.benlysta.com.

18. Will there be any patient assistance programs available for BENLYSTA?
Yes, Human Genome Sciences and GlaxoSmithKline will be offering a Patient Assistance Program (PAP.) For individuals who are uninsured and meet eligibility requirements the PAP will provide the medication free of charge. For commercially insured and underinsured patients who are unable to afford the cost-share with BENLYSTA, and

do not qualify for the PAP, BENLYSTA Gateway can help determine eligibility for assistance. For more additional information and questions about eligibility call BENLYSTA Gateway at 1-877-4-BENLYSTA, or visit benlysta.com.

19. Are there other treatments being researched for lupus?
There are a number of pioneering biotechnology and pharmaceutical companies involved in the research and development of new therapies for lupus, and there are several promising treatments in the near-term pipeline. The historic decision by the FDA will likely stimulate further investment in additional clinical trials in lupus. We can't make new treatments a reality without your support. You can help by learning more about clinical trials and volunteering to participate in a clinical trial. To learn more, visit the LFA's Center for Clinical Trial Education at www.lupus.org/clinicaltrials.

20. Why has it taken so long to find a treatment for lupus?
Lupus is a complex disease. It can affect multiple organ systems and symptoms can range in severity from one day to the next. Also, lupus affects each person differently, with varying responses to treatment. The complexity and heterogeneity of the disease presents challenges in evaluating potential new therapies. With each research study, regardless of the outcome, there are new discoveries that help pave the way for new therapies.

21. What is the LFA doing to further the development of new treatments for lupus?
As part of the LFA's **National Research Program: *Bringing Down the Barriers*™**, the LFA has recently launched new initiatives that will standardize and improve clinical trial design, allowing future studies to be completed successfully. These initiatives include a new worldwide Lupus Research Registry through the LFA's *Center for Clinical Trials Education*, and a Web-based service to standardize research training on instruments used to assess disease activity in clinical studies and practice. The LFA also is partnering with key stakeholders from industry, government, and the scientific community to evaluate data from previous lupus clinical trials with the goal to improve the design of future studies. These initiatives, along with the approval of BENLYSTA, will help provide a pathway toward additional treatments specifically developed for lupus.