Clinical Trial Results

Study to Evaluate BIIB059 in Cutaneous Lupus Erythematosus (CLE) With and/or Without Systemic Lupus Erythematosus (SLE) (LILAC)

- Study Drug: BIIB059
- Condition: SLE/CLE
- Protocol #: 230LE201
- Study #: NCT02847598
- Study Dates:
  - » Start: 20 Oct 2016
  - » End: 18 Nov 2019

Overview

Thank You!

A clinical study participant belongs to a large community of clinical research participants around the world. By participating in a study, they help researchers answer important health questions and evaluate new medical treatments.

The clinical study for the drug helped researchers learn more about BIIB059 safety and if BIIB059 might help people with Systemic Lupus Erythematosus (SLE) or Cutaneous Lupus Erythematosus (CLE). Both SLE and CLE are long-term diseases. In both SLE and CLE, the body's defense system against infection makes a mistake and attacks healthy cells. This can affect many parts of the body, especially the joints and skin.

Biogen, the sponsor of this study, thanks those who participated and believes it is important to share the overall results of the study. If you have questions, please speak with the doctor or staff at the study research center.

Why was this study needed?

The study was done to see whether BIIB059 reduced symptoms in participants with Systemic Lupus Erythematosus (SLE) and/or Cutaneous Lupus Erythematosus (CLE).

General Study Information

SLE can affect many parts of the body. Some of the most common symptoms are rash, swelling of the joints, swelling of the thin layer of tissue surrounding organs, and swelling of the kidneys. SLE can have phases where symptoms are active, called flares. It can also have periods where it causes only mild symptoms, called remissions. An early sign that someone might develop SLE is a related skin disease called Cutaneous Lupus Erythematosus (CLE).

CLE is a disease caused by a person's immune system attacking their skin. Many, but not all people with CLE also have SLE. In CLE, the attack causes rashes or sores which can be painful and itch. The rashes and sores usually appear on skin that is exposed to sunlight on a regular basis, such as the face, scalp, neck, arms, and legs. CLE can cause scars, hair loss, and changes in skin color where the rashes or scars appear.

In this study, researchers studied a drug called BIIB059. They wanted to see if BIIB059 would work better than a placebo. A placebo looks like the study drug but contains no real medicine. Using a placebo helps researchers learn if the study drug works.

There were two questions that researchers wanted to answer:

- Part A – Did BIIB059 reduce symptoms in participants with SLE, especially tender or swollen joints?
- Part B – Did BIIB059 reduce symptoms in participants with CLE?

Who Took Part in the Study? *(Lists are not comprehensive)*

Part A and Part B

- Were between 18 and 75 years old
- Did not have a history of infections during the 12 months before the study
- Did not take certain medications during the 12 weeks before the study
- Did not have certain types of kidney disease

Part A

- Diagnosed with SLE
- Had at least
  - » 4 swollen joints in their hands and wrists, and
  - » 4 tender joints
- Had active skin problems due to SLE

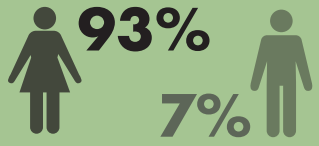
Part B

- Diagnosed with CLE by lab testing
- Had active skin problems

Study Locations

Part A

Participants were mostly female, with an average age of 40 years old.



18	40	73
Youngest Age	Average Age	Oldest Age

Part B

Participants were mostly female, with an average age of 44 years old.

18	44	66
Youngest Age	Average Age	Oldest Age

What Happened During the Study?

The study started October 20th, 2016 and ended on November 18th, 2019. There were 264 adults who participated in the study. Participants had to have had SLE and/or CLE. They answered questions about their medical history before the study began. When the study ended, the sponsor created a report of the results. This is a summary of that report.

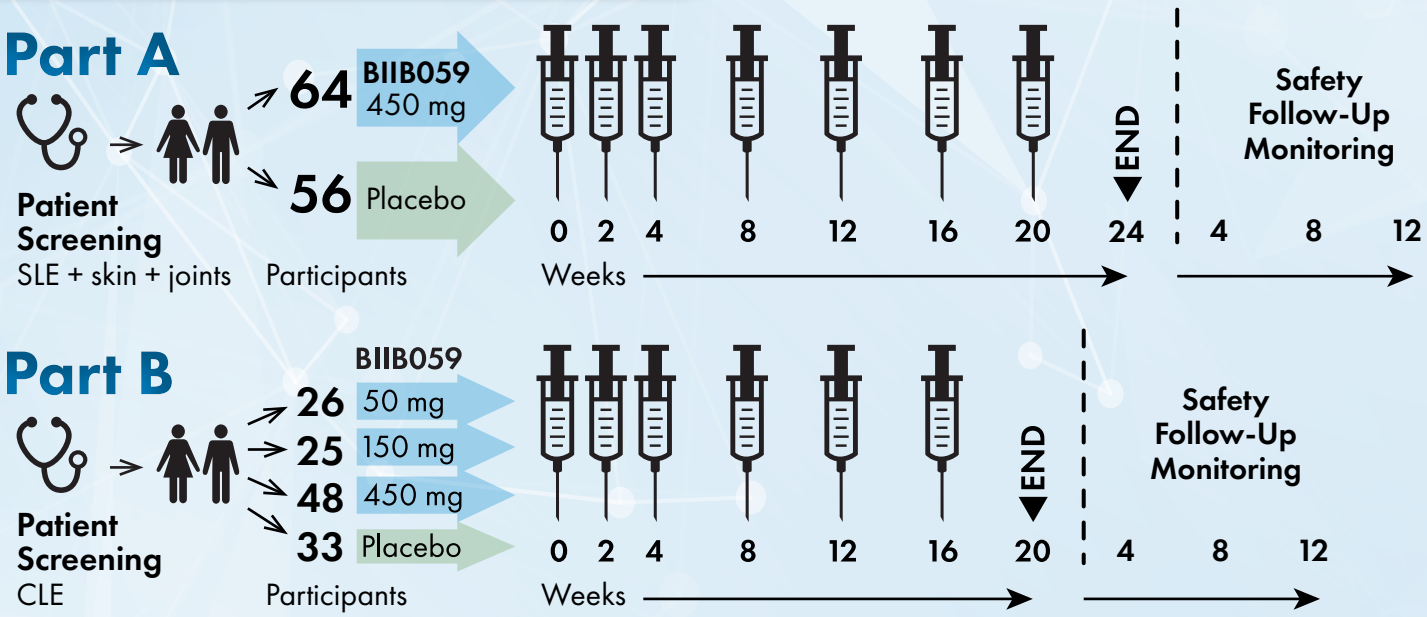
Participants were put into groups at random to reduce differences between the groups.

The study was "double blinded." This means that people involved in the study did not know who was receiving BIIB059 or placebo. This included participants and researchers. Most participants were able to stay on their regular treatments while taking part in this study.

Researchers followed participants for an additional 12 weeks after treatment ended.

The doses and number of participants for each group in Part A and Part B are shown in the figures below.

How was the study done?



Part A Study Design

Participants were put into 1 of 2 different groups. One group received 450 mg of BIIB059 and the other group received a placebo. There were 12 other participants who received lower doses of BIIB059. All participants received the treatment by an injection under the skin. They received injections once every 4 weeks for 20 weeks. One extra dose was given 2 weeks after the first dose for a total of 7 doses.

Part B Study Design

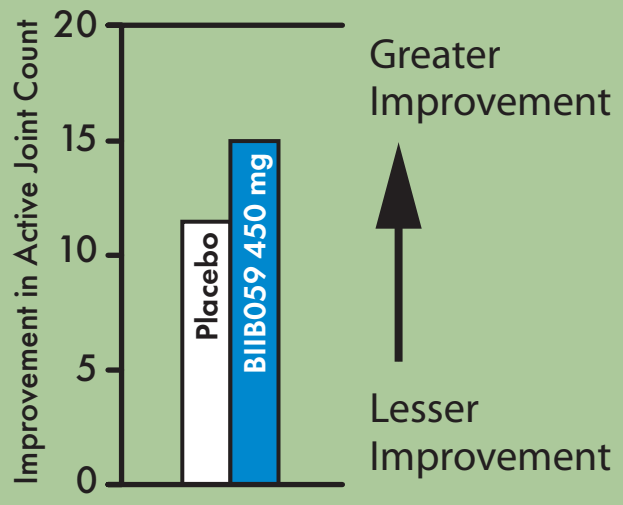
In Part B of this study, participants were put into 1 of 4 different groups. One group received a placebo. The other three groups received 50 mg, 150 mg, or 450 mg doses of BIIB059. All participants received the treatment by an injection under the skin. They received injections once every 4 weeks for 12 weeks. One extra dose was given 2 weeks after the first dose for a total of 5 doses.

What Were the Study Results?

A Did BIIB059 reduce symptoms in participants with SLE, especially tender or swollen joints?

Yes. Researchers used a standard checklist. The checklist evaluated 28 joints for pain and swelling. If a joint had pain or swelling, it was an "active joint". The checklist score was recorded for participants at the beginning and end of the study. To understand if BIIB059 helped participants, the results were compared when the study was over. The researchers wanted to see if joint pain and tenderness improved with BIIB059.

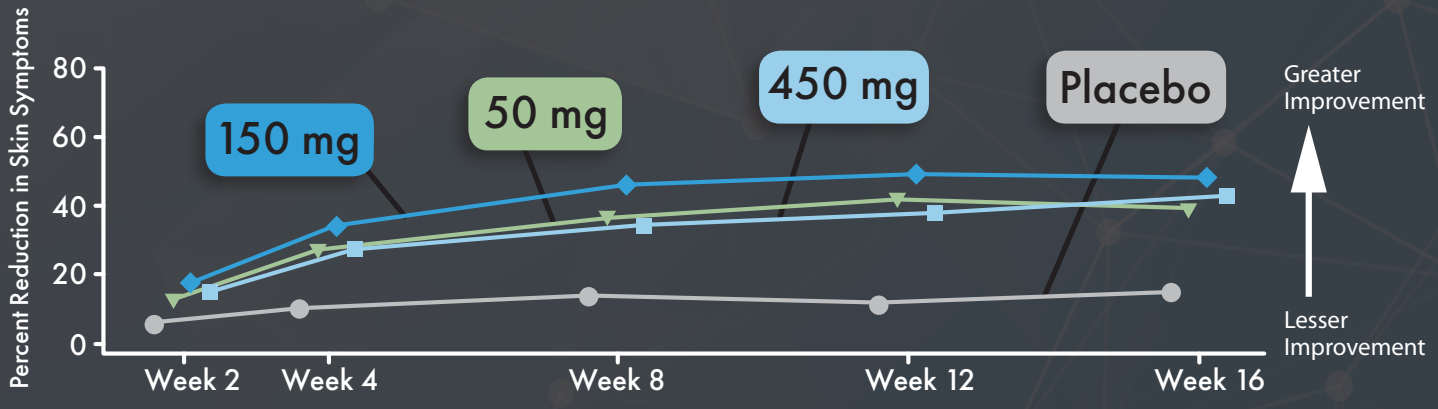
- Researchers found that there was a difference in checklist scores between the two groups.
- Those who took BIIB059 had between 3 and 4 fewer painful or swollen joints than those who took placebo at 16 weeks.



B Did BIIB059 reduce symptoms in participants with CLE?

Yes. Researchers used a CLE standard symptom checklist. The checklist evaluates rashes and other skin symptoms. The checklist allowed researchers to give each participant a score for how severe their skin symptoms were. Their scores were recorded at the beginning and at the end of the study.

- Researchers found that there was a difference in scores between the four groups.
- Those given BIIB059 showed between a 39% to 48% improvement in their skin symptoms at week 16. The amount of improvement depended on the dose of BIIB059.
- The group given placebo showed an average of 14% improvement.



How Many Adverse Events Occurred?

Research is needed to know whether a drug causes medical problems. These medical problems are called adverse events. When drugs are being studied, researchers keep track of all adverse events. Researcher also record any adverse events that happen after the study is done. Not everyone experiences them. Adverse events may or may not be related to the study drug.

One goal of this study was to learn more about the potential adverse events of BIIB059.

How many participants had adverse events in each group?

Part A

	Placebo (out of 56 Participants)	BIIB059 (out of 76 Participants)
How many participants had adverse events?	38 (68%)	45 (59%)
How many participants had serious adverse events?	6 (11%)	4 (5%)
How many participants stopped treatment because of adverse events?	3 (5%)	2 (3%)

Part B

	Placebo (out of 33 Participants)	50 mg BIIB059 (out of 26 Participants)	150 mg BIIB059 (out of 25 Participants)	450 mg BIIB059 (out of 48 Participants)	All taking BIIB059 (out of 99 Participants)
How many participants had adverse events?	22 (67%)	18 (69%)	15 (60%)	38 (79%)	71 (72%)
How many participants had serious adverse events?	3 (9%)	1 (4%)	3 (12%)	3 (6%)	7 (7%)
How many participants stopped treatment because of adverse events?	0	3 (12%)	1 (4%)	4 (8%)	8 (8%)

What Were the Adverse Events?

What were the most common adverse events?

Headache
Placebo: 3 of 33 (9%)
BIIB059: 9 of 99 (9%)

Common Cold
Placebo: 2 of 56 (4%)
BIIB059: 5 of 76 (7%)

Common Cold
Placebo: 2 of 33 (6%)
BIIB059: 10 of 99 (10%)

Upper Respiratory Tract Infection
Placebo: 1 of 33 (3%)
BIIB059: 6 of 99 (6%)

SLE*
Placebo: 4 of 33 (12%)
BIIB059: 7 of 99 (7%)

Diarrhea
Placebo: 3 of 56 (5%)
BIIB059: 5 of 76 (7%)

Urinary Tract Infection
Placebo: 4 of 56 (7%)
BIIB059: 5 of 76 (7%)

Injection Site Redness
Placebo: 1 of 33 (3%)
BIIB059: 9 of 99 (9%)

Joint Pain
Placebo: 2 of 33 (6%)
BIIB059: 6 of 99 (6%)

Part A **Part B**

*This includes people who already had SLE but experienced a flare.

What Were the Adverse Events? (Continued)

What serious adverse events did participants have?

There are certain times an adverse event is serious. These are when they require hospital care or cause lasting problems. Adverse events are also serious when they are life-threatening or cause death. This is a list of serious adverse events (SAEs) in participants taking BIIB059.

Part A

- 4 out of the 76 (5%) participants taking BIIB059 had SAEs
- In placebo group, 6 out of 56 (11%) had SAEs
- There were 3 deaths; all deaths were in participants who received placebo
- SAEs experienced by participants taking BIIB059:
  - Ankle fractures
  - Ataxia (loss of muscle control)
  - Epilepsy
  - Headache
  - Blood clot in the lung
  - Incarcerated umbilical hernia (tissue or part of intestine pushes through abdomen near belly button and can't be pushed back into place)

Part B

- 7 out of 99 (7%) participants taking BIIB059 had SAEs
- In the placebo group, 3 out of 33 (9%) had SAEs
- No deaths occurred
- SAEs experienced by participants taking BIIB059:
  - SLE
  - Heart attack
  - Abnormally fast heartbeat
  - Skin cancer
  - Non-cancerous tumor in uterus
  - Anemia
  - Headache
  - Allergic reaction to BIIB059
  - Blocked intestines
  - Tingling in hands and/or feet
  - Vision problems
  - Blood poisoning from a urinary tract infection

Where Can I Learn More About the Study?

You can find more information about the study online at www.clinicaltrials.gov. Once on the site, type NCT02847598 into the search box and click "**Search**". You can also find more information online at www.clinicaltrialregister.eu. Once on the site, click "**Home & Search**", then type 2015-004359-32 in the search box and click "**Search**".

If you have questions about BIIB059 or the results of this study, please speak with the doctor or staff at the study research center.

Official Study Title: Study to Evaluate BIIB059 in Cutaneous Lupus Erythematosus (CLE) With or Without Systemic Lupus Erythematosus (SLE) (LILAC)

Biogen, the sponsor of this study, has its headquarters in Cambridge, Massachusetts (USA).

The results presented here are for a single study. You should not make changes to your therapy based on the results without first consulting your doctor. Further studies with BIIB059 are ongoing.

US Clinical Study Database

- https://www.clinicaltrials.gov/ct2/show/NCT02847598
- www.clinicaltrials.gov
- Study#: NCT02847598

EU Clinical Study Database

- https://www.clinicaltrialsregister.eu/ctr-search/search?query=2015-004359-32
- www.clinicaltrialsregister.eu
- Study#: 2015-004359-32

Thank You!

Clinical trial participants help doctors and researcher advance medical knowledge and develop new treatments for patients. Biogen would like to thank all the participants and caregivers involved in this study. We hope this summary helps you better understand your role in helping researchers learn more about the use of BIIB059 in patients with SLE and/or CLE.

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