Alexion NMO Clinical Trial Webinar

7:00 a.m. PST, 10 a.m. EST

Friday, 12/12/14

The PREVENT Study

Webinar Format

- Session is 45 minutes in length (Welcome, Intro, Presentation, Q&A)
- Attendees may submit questions in the Q&A window online - please be as succinct as possible if you choose to submit a question
- After the presentation is complete, the presenters & panelists will address as many questions as time allows
- Thank you for understanding that not all questions will be addressed
- Webinar slides and audio will be posted on the foundation website for later viewing / audio

Presenter

Richard Riese, M.D.
Global Medical Science Lead
Alexion Pharmaceuticals

Panelist

Brian Weinshenker, M.D.
Expert NMO Clinician
Mayo Clinic

Moderator

Michael Yeaman, Ph.D.
Advisor, The Guthy-Jackson Charitable Foundation

Presentation Format

- Content is solely that of the respective industry or its representative
- Presenters are afforded a maximum of 15-20 minutes total time
- Questions / answers are then afforded up to 20 minutes of time
- The webinar is being recorded for purposes of future distribution
- All perspectives are offered only for stakeholder self-education
- The Guthy-Jackson Charitable Foundation does not perform clinical trials nor does it endorse any particular clinical trial design or drug

THE PREVENT STUDY

Prevention of
RElapses and
EValuation of
Eculizumab in
Neuromyelitis
OpTica

Presented by Richard Riese, MD
Global Medical Science Lead
Alexion Pharmaceuticals

THE PREVENT STUDY in Relapsing Neuromyelitis Optica (NMO)

Study goal

- This research is studying the ability of a medication called eculizumab to prevent relapses in relapsing neuromyelitis optica (NMO) and NMO-Spectrum Disorder (SD)

Study medication: eculizumab

- Eculizumab is a monoclonal antibody that blocks the immune system's attack on healthy cells, which in turn may prevent relapses (attacks)
- Eculizumab is not currently approved for the treatment of NMO, but has been approved to treat two other rare diseases in some countries

THE PREVENT STUDY: Eculizumab in Relapsing NMO

Previously tested with 14 Relapsing NMO patients

12 out of 14 patients did not experience relapses for the year they were treated with eculizumab

The goal of this study is to evaluate safety and potential efficacy of eculizumab in a larger group of patients

The study will recruit up to 132 patients in sites in 20 countries around the world (Asia, Australia, Europe, North and South America)

You may eligible to participate in this study if you are an adult 18 yrs or older and:

Diagnosis

- You have been diagnosed with relapsing neuromyelitis optica (NMO) or NMO-spectrum disorder (NMO-SD)
- Have had a positive blood test for the NMO antibody (AQP4)

Relapse History

- At least 2 relapses in the last 12 months (can include the first attack that led to initial diagnosis)

Or

- Experienced 3 relapses in the past 24 months
  - With at least 1 attack or relapse in the last 12 months

You may not be able to participate in this study if, prior to screening:

- You have been taking rituximab (Rituxan®) for the past 3 months
- You have been taking mitoxantrone for the past 3 months
- You have had IVIg within the past 3 weeks

Once you are eligible, what to expect

If you are eligible to participate, you will be randomly assigned to one of 2 groups– one group will receive the active study medication (eculizumab) and one will receive an IV without active medication (placebo)

- The study coordinators, the physician or nursing staff will not know whether you have received the active study medication or placebo
- Since this is an **"add on study,"** you be will be able to continue to take your regular medication for NMO
- All patients who participate in this study will be eligible to participate in a follow up study in which all participants will receive eculizumab

Both groups will be given intravenous infusion– however, one group will receive the active medication (eculizumab) and one will receive an infusion without active medication (placebo)

Stages of The Study

1. Screening:
The period when your doctor and the study doctors will determine if you are eligible for the study (1 to 3 weeks).

2. Treatment:
You will receive eculizumab or placebo (both by intravenous infusion which is given in the study clinic) , every week for the first 5 weeks and then every 2 weeks afterwards. The treatment phase of the study will last potentially as long as 2 years.

3. Relapse or Attack:
If you have a relapse, your doctor will treat you.
The study will end for you, but you will be monitored for an additional 6 weeks.

4. Follow up Period:
Afterwards, you may be eligible to continue in another study--an extension study--where every patient will receive eculizumab. The follow up study could last for up to 4 years.

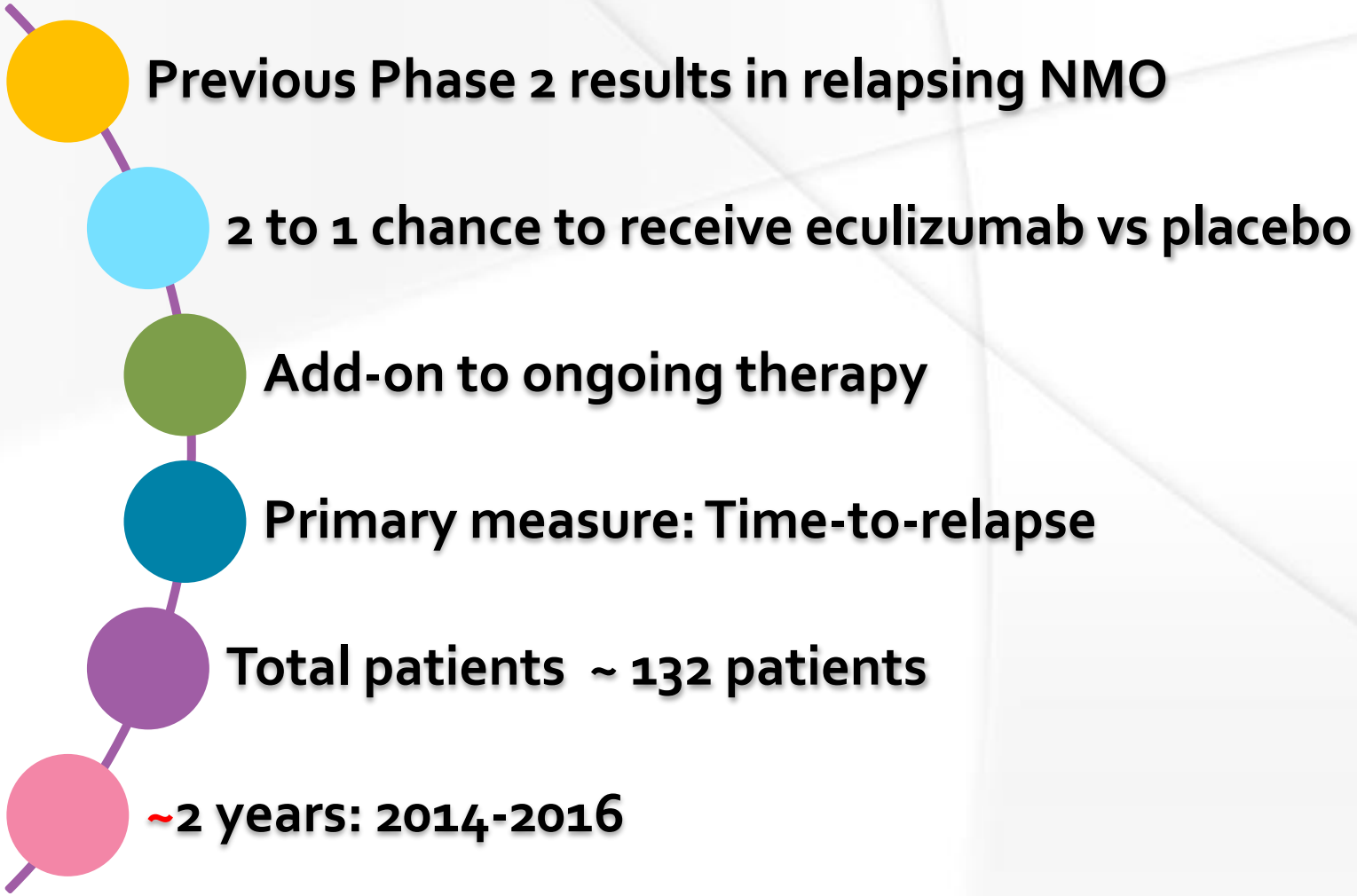
Your doctor can help determine if you are eligible and help you decide whether to participate

Risks and Side Effects

- **Known side effects: over 10 years experience with eculizumab**
- **Most frequently reported adverse reactions in years of use in:**
  - Paroxysmal Nocturnal Hemoglobinuria (PNH), a rare red blood cell disease: Headache, nasopharyngitis (inflammation of the nose and throat), back pain, nausea
  - Atypical Hemolytic Uremic Syndrome (aHUS), a rare genetic disorder: Upper respiratory tract infection, diarrhea, headache, anemia, vomiting, nausea, urinary tract infection, decreased white blood cell count
- **Risk of meningococcal Infection:**
  - One patient had meningococcal sepsis in the Phase 2 NMO study with full recovery
  - Meningococcal vaccine is required for entry into the study

Most frequent side effects in Phase 2 NMO study
Headache
Nausea
Dizziness
Coughing
Diarrhea
Abdominal pain
Rash

Summary of The PREVENT STUDY



More Information

To learn more about clinical trials being performed by Alexion

you are invited to contact Alexion at: clinicaltrials@alxn.com

Your opinion on patient education materials to encourage clinical trial participation:

To provide your opinion / feedback on patient education materials about clinical trial participation, being fielded by

a third party market research group ,Rare Patient Voice

you are invited to contact : wes.michael@rarepatientvoice.com

Note: GJCF has no role in the development of these clinical trial education materials

Questions

Learn More

This webinar will be hosted on the foundation website at:

http://nmotion.guthyjacksonfoundation.org/series-one-nmo-clinical-trial-update/

GUTHY JACKSON® Charitable Foundation

Conclusion

- Content is solely that of the respective industry or its representative
- Webinar slides and audio will be posted on the foundation website for later viewing/audio
- All perspectives are offered only for stakeholder self-education
- The Guthy-Jackson Charitable Foundation does not perform clinical trials nor does it endorse any particular clinical trial design or drug
- We hope this webinar has been informative to all stakeholders