



Small



Madara

SPOTLIGHT ON CLINICAL RESEARCH

Amyloid Cardiomyopathy & Polyneuropathy Rx, MRIs in Patients with Abandoned Endocardial Leads

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Editor's note: This is the eighth in a series of articles from the Penn Medicine Lancaster General Health Research Institute that describes ongoing research studies. Other active studies have been described in previous issues of this Journal.

Physicians who wish to refer patients for any of the studies mentioned below are encouraged to contact the Penn Medicine Lancaster General Health Research Institute at 717-544-1777.

Other members of the Penn Medicine LGH staff who are conducting research and wish to have their studies described here are encouraged to contact the offices of JLGH at 717-544-8004.

SPONSORED STUDIES

CARDIO-TTRANSFORM:

A Study of Patients with Transthyretin-Mediated Amyloid Cardiomyopathy (ATTR CM)* to Evaluate the Efficacy and Safety of AKCEA-TTR-LRx.

Sponsor: Ionis Pharmaceuticals

Principal Investigator: Tareck Nossuli, M.D.

This multicenter, double-blind study to evaluate the efficacy of AKCEA-TTR-LRx was developed by Ionis Pharmaceuticals in collaboration with Akcea Therapeutics. This drug is a second-generation RNA-targeted therapy designed to inhibit TTR production. Participants will be randomized to receive either the study drug, AKCEA-TTR-LRx, or placebo by subcutaneous injection. Due to the high risk that the study drug lowers Vitamin A levels, participants in both groups will also receive a Vitamin A supplement while in the study.

Dr. Nossuli and the study team at the Research Institute will regularly check in with study participants for the 36 month duration of the study. The total study in all centers will enroll up to 750 patients; LGH plans to enroll up to 10 patients.

OPTIMIZER PAS:

Post-Approval Study (PAS) of the OPTIMIZER Smart Device and Cardiac Contractility Modulation (CCM) Therapy.

Sponsor: Impulse Dynamics

Principal Investigator: Matthew Bernabei, M.D.

This post approval study will examine the long-term outcomes in patients who received the OPTIMIZER Smart device and cardiac contractility modulation (CCM) therapy. CCM Therapy improves cardiac contractility by applying presystolic electrical cardiac stimulation via a pacemaker-like device. The 20 participants LGH plans to enroll must have NYHA functional class III symptoms and a left ventricular ejection fraction of 25%-45% in order to be eligible for this non-randomized, single arm open label study. Study participants follow standard of care post-implant, but they also agree to annual safety assessments to measure their health outcomes and quality of life. The unique use of home healthcare providers to carry out some assessments decreases the study's impact on participants' lives. LGH received approval to begin this study in September of 2020 and has already enrolled its first patient.

ALN-TTR-02:

A Multicenter Observational Study to Evaluate the Effectiveness of Patisiran in Patients with Polyneuropathy of ATTRv Amyloidosis with a V122I or T60A Mutation.

Sponsor: Alnylam Pharmaceuticals

Principal Investigator: Roy Small, M.D.

This phase 4 study aims to determine the effectiveness of the drug patisiran in patients diagnosed with hereditary transthyretin-mediated amyloidosis (ATTRv)

* For a detailed discussion of amyloid cardiomyopathy, see the article by Dr. Roy Small in the Fall 2020 issue. Small R. Amyloid Cardiomyopathy: An update. *J Lanc Gen Hosp.* 2020;15(3):85-89. http://www.jlgh.org/Past-Issues/Volume-15-Issue-3/Small_Amyloid-Cardiomyopathy.aspx

with polyneuropathy who have a mutation of genes V122I or T60A. Patients are not excluded if they were already prescribed or already take patisiran. The study is divided into three groups: Prospective, Mixed, and Retrospective. These groups enable the study to capture data from individuals all along the spectrum of treatment. The Prospective group enrolls patients who have not yet started patisiran but plan to. The Mixed group enrolls patients who have taken patisiran for less than 12 months. Finally, the Retrospective group enrolls patients who have taken patisiran for over 12 months.

The primary focus of this research is to measure the impact of patisiran on participants' polyneuropathy disability score, and their overall quality of life. Since the study includes patients already prescribed or taking patisiran and follows standard of care, the risks are minimal to study participants. This patient population is limited in this demographic, but LGH plans to enroll at least one patient into the study.

INVESTIGATOR-INITIATED STUDIES

EVALUATING MRI SCANNING

Evaluating MRI Scanning in Patients with Fractured or Abandoned Endocardial Leads

Principal Investigator: Sandeep Bansal, M.D.

Authored by: Douglas Sell

The Lancaster General Health Research Institute, The Heart Group, and The MRI Group are conducting a research study of patients with cardiac implanted electronic devices (CIED) and fractured or abandoned leads to further determine the safety and efficacy of MRI scanning in this patient population. Currently, the Center for Medicare Services (CMS) has not approved MRI in such patients and participants may be responsible for whatever costs are not covered by their insurance carriers. Though patients with abandoned leads will have the opportunity to get an MRI in this study, study participants or their insurance company will be responsible for the cost of the MRI and device interrogations required pre and post MRI procedure.

The MRI ordering process for this study does have an additional communication step. After placing the MRI order for a patient with an abandoned lead, a research coordinator will contact both the patient and ordering physician to explain the research study option and review patient financial obligations. The physician and patient will then discuss and determine participation in the MRI abandoned lead study. The ordering physician will also be surveyed to assess how the availability of MRI in this patient population impacted patient care.

If you have any questions on the study, you may contact Dr. Sandeep Bansal at 717-544-8382 or Clinical Research Coordinator Andrew Hershey, R.N., at 717-544-1412.

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