



News and Stories - Fall 2021

Is CRISPR Therapy a cure?

The latest research study, led by Dr. Julian Gillmore and published in the New England Journal of Medicine, demonstrated that CRISPR gene-editing technology may be able to provide hATTR amyloidosis patients substantial relief.

Dr. Gillmore concentrated on studying hATTR amyloidosis. This form of the condition is often associated with heart failure, as the nerves of the heart are typically affected.

The condition places a burden on patients, caregivers,

and children to who the condition could be passed in the future.

This study aimed to evaluate whether CRISPR could improve outcomes. It involved 6 patients who were diagnosed with hATTR amyloidosis. Three of the individuals were given one infusion of NTLA-2001, a low dose of CRISPR. The last 3 patients were given a higher dose of the same treatment. The hope was that this therapy would lower the levels of TTR in the bloodstream. This is exactly

what they found. At the 1-month mark, TTR was lowered significantly. Those with the higher dose of the treatment decreased TTR levels by 87% on average. The low dose group experienced a decrease of 52% on average.

These results are particularly meaningful because they demonstrate that one single infusion can improve patient outcomes dramatically.

Although there is much more research to be done on this therapy in hATTR amyloidosis, the results so far are very promising. **AF**

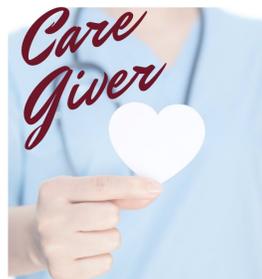
Caregiver Binder

The binder was designed by the AF staff, a team that strives to make caregiving easier for caregivers and patients.

Each section has links to PDF files that are designed so that you can print your own copy and keep it in a 3-ring binder.

(<https://amyloidosis.org/resources/#caregiver-resources-binder>).

Glenn M, an amyloidosis patient said "Thanks for the handsome amyloidosis



binder—I bring that to all of my appointments now. Very handy for the overwhelming amount of information and note taking with my numerous providers".

If you do not have access to a printer, we will mail you an assembled binder. Just email your request for a binder to: info@amyloidosis.org. **AF**

Education • Awareness • Support • Research

www.amyloidosis.org



Will You Help Us Plan for the Future?

People give their time, talents, and treasures to non-profit organizations because they believe in the mission of the organization and they understand the importance of preserving the programs and services offered.

In the case of the Amyloidosis Foundation, the mission is to fund research, create awareness in the medical community and to provide support through patient education and advocacy programs.

By making us a beneficiary of your will

or revocable trust, you'll help guarantee our financial strength tomorrow, without affecting your cash flow or your family's financial stability today.

The idea of making a gift may feel intimidating. Actually, a charitable gift is simple and easy to set up than many people realize.

By making a bequest to the Amyloidosis Foundation, you will join many who have decided that our mission is important and that supporting our cause in

the future is imperative.

What is a BEQUEST?

Will bequests are the most popular and personal way to making a difference beyond your lifetime.

A bequest is a charitable gift of property to be delivered at the donor's passing. You can create a bequest simply by directing in your will that certain money or property be transferred from your estate to the Amyloidosis Foundation.

(Continued on page 7)

Patient Resources

The foundation has several programs that benefit patients and their families. All of these are provided free of charge.

- Webinar recordings posted on our website
- Updated informational pamphlets
- Listing of experienced physicians that specialize in amyloidosis. Email us anytime with questions: info@amyloidosis.org
- Treatment Centers (US / International)
- Support Groups
- Newsletters
- Webinars
- Caregiver/Patient Binder
- Fundraising Toolkits

Follow Us!



Stay connected for all the latest information on Amyloidosis:

Web: www.amyloidosis.org
 Twitter: [@Amyloidosisfdn](https://twitter.com/Amyloidosisfdn)
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 Instagram: [@amyloidosisfoundation](https://www.instagram.com/amyloidosisfoundation)





President's Corner

The holidays are right around the corner and we at the Amyloidosis Foundation are thankful for your continued support this year. Our community is a family, and even though we are from different parts of the world, we keep pushing forward toward a cure.

It's a powerful thing to talk to patients on the phone and receive messages via social media and hear your stories. Our journeys are all different, yet have a familiar ring to them. We appreciate each and every one of our donors that make our work possible, the work of supporting patients, education, families and research.

We wish you and yours a Happy Holiday season and a healthy New Year!



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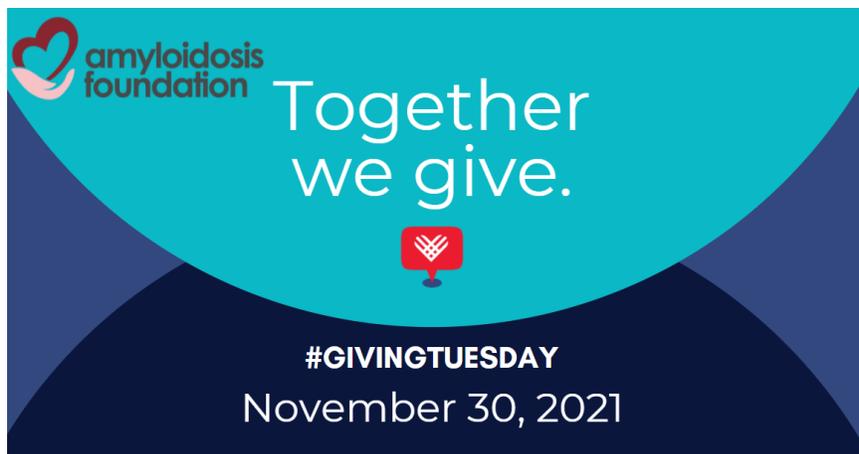
Giampaolo Merlini, MD

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Save The Date – 11.30.21



Our newsletter is published quarterly (Spring, Summer, Fall and Winter) by the **Amyloidosis Foundation**. We welcome letters, articles and suggestions.

Please contact us anytime at: **info@amyloidosis.org**, (248) 922-9610
or 7151 N. Main Street, Ste. 2, Clarkston, MI 48346

If you wish to receive an electronic version, please send us an email:

info@amyloidosis.org



You've Seen Them Care, Now Show Your Care About Them

November is National Caregivers Month, here are some ideas to give thanks!

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1 Bring her a low-maintenance houseplant

2 Take in his mail

3 Shovel snow from her driveway and walkway

4 Grab his grocery list and go shopping

5 Arrange a play-date for her kids



6 Gas up his car

7 Encourage her to relax with a gift of bubble bath

8 Do a load of his laundry

9 Take her kids or grandkids to a movie

10 Do some holiday shopping for him



11 Send her flowers

12 Bring over a guitar and sing familiar songs



13 Stop by with a board game to play

14 Take him out for coffee

15 Teach her a skill she admires you for



16 Visit him with a pet that has a sweet disposition



17 Send her to a day spa for some pampering

18 Buy him a coupon for exercise classes

19 Take her dog on a hike

20 Drop off his packages at the post office



21 Give her a foot massage

22 Water his flowers

23 Do her dishes



24 Bring a favorite movie and watch it together

25 Return his library books

26 Mow her lawn



27 Wash his car

28 Arrange for a housecleaning service to visit



29 Cook and deliver a meal he likes

30 Clean her gutters

31 Replenish his coffee or tea supply



32 Bring ingredients and cook a meal with her



Fundraising on Facebook is Easy, Give it a Try!

Have you ever donated online to a fundraiser? For a cause or group that you feel strongly about supporting? Maybe a friend or family member has sent you a donation link, to share details for an event or asked you to help? Well, here is your chance to make a difference.

Since 2017, the Amyloidosis Foundation has had great online support from friends and family members — starting fundraisers in honor or memory of a loved one, their own birthday or anniversary. Last year, during

a pandemic, we raised a whopping \$99,208 — with each fundraiser averaging almost \$300! Crowd funding has grown dramatically over the years, and we are thankful for our generous donors. Next time you are on FB, use the details below to start your own fundraiser!

Support A Cause You Care About! Create a fundraiser to raise money for the Amyloidosis Foundation on Facebook.

**Starting is easy**

We'll help you get started and give you tips to reach your goal.

**Share with friends**

Connect friends to your cause and gain supporters across Facebook.

**Get donations**

People can donate to your fundraiser in just a few clicks without leaving Facebook.



<https://www.facebook.com/fund/amyloidosisfdn/> **AF**

Did you miss our Fall Webinar?

Heather Landau, MD, is a hematologist-oncologist and the Director of the Amyloid Program at Memorial Sloan Kettering (MSK) Cancer Center focused on increasing access to novel, effective therapies in AL amyloidosis and has investigated drugs at all stages of development.

She has conducted multiple investigator-initiated clinical trials exploring novel agents and approaches, including consolidation and maintenance strategies in high-risk patients. She led the development of MSK's Outpatient Transplant Service and is

studying innovative ways to optimize high-dose therapy and transplant care.

Pharmacokinetically dosed conditioning has the potential to truly personalize high-dose therapy and Dr. Landau has shown that delivering transplant care at home is safe and improves the quality of life and satisfaction of patients and caregivers.

Dr. Landau has worked with the FDA to identify scientifically rigorous but feasible clinical trials designs and incorporate clinically meaningful endpoints to accelerate drug approval in this disease.

Partnering with colleagues in pathology and clinical laboratory medicine, Dr. Landau is developing more sensitive methods of detecting and monitoring monoclonal disease using mass spectrometry.

To view the webinar:

<https://bit.ly/2021FallWebinar>

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We Need YOU to Share Your Patient Story

How did it feel when you or a family member were first diagnosed with amyloidosis? What did you most want to hear about from another patient who was going through the same thing?

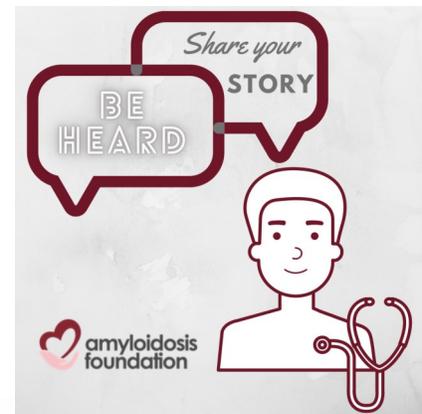
On our website and in past newsletters, we've shared many patient stories, about various types of amyloidosis. Now we are asking you to be brave and show how you are managing your disease,

what tips you have, how you keep track of symptoms, doctor appointments and more.

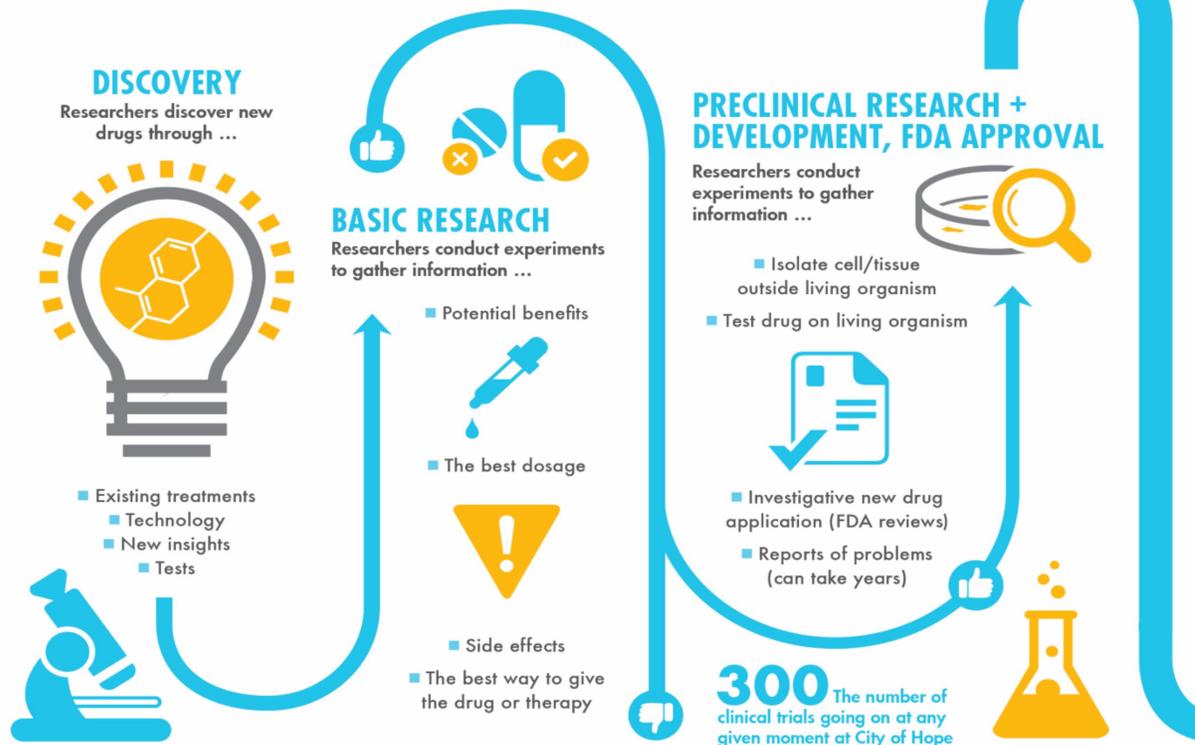
Show your support for others who are fighting this rare disease by writing your story, providing hope, encouragement and empathy.

Send your story and a few photos to us at: kathi@amyloidosis.org. Feel free to call us with any questions—we look

forward to hearing from you and know that other patients are waiting to read your story. **AF**



BEHIND THE SCENES OF A CLINICAL TRIAL





Will You Help Us Plan For the Future?

(Continued from Page 2)

Bequests from wills vary widely...some are a few hundred while others can range from a few hundred thousand to millions of dollars. Every bequest is equally appreciated.



Why make a BEQUEST?

Charitable bequests are an excellent way to pass on assets that may otherwise be substantially taxed. The donor's estate may be entitled to an estate tax charitable deduction for the full, fair market value of the

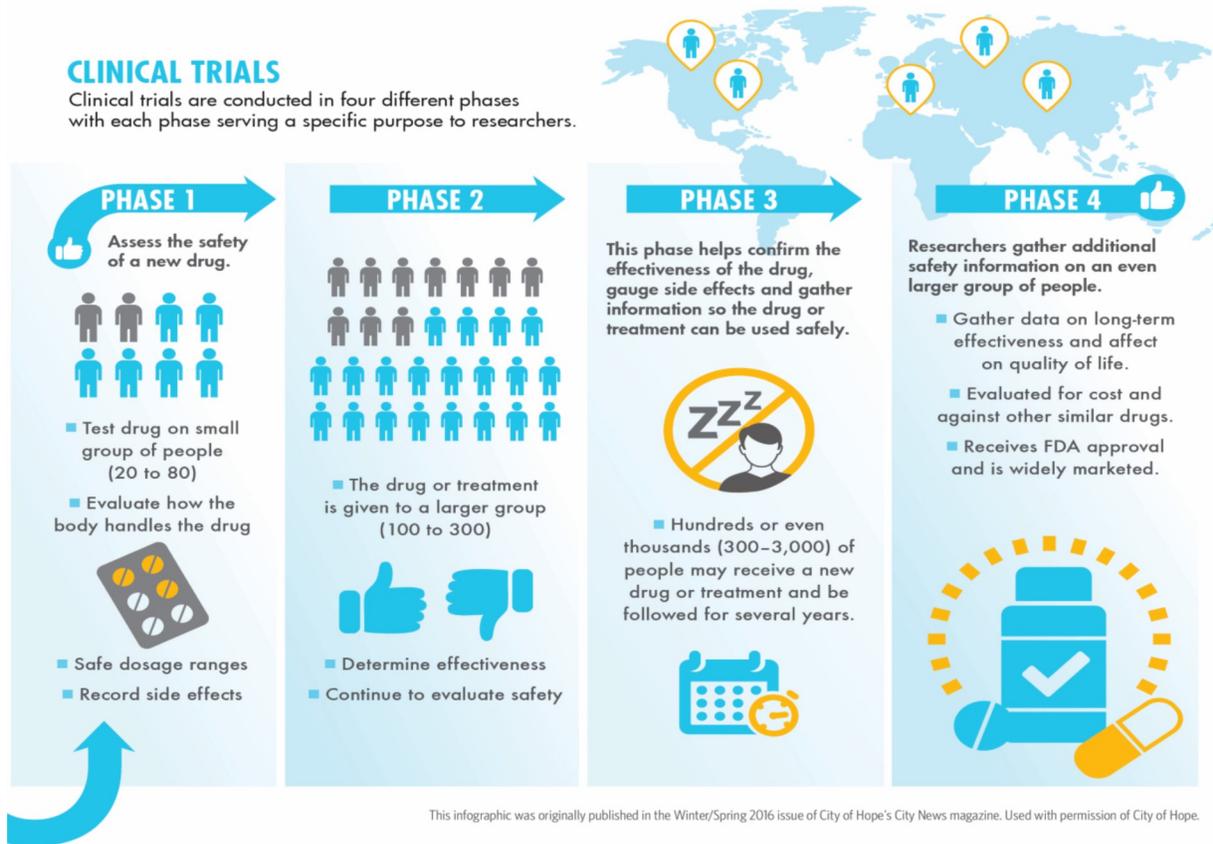
bequest. Also, wills are fully revocable before death.

There are many creative, long-term giving opportunities and we would be happy to talk through the options with you.

We are here to help every step of the way. We encourage you to consult with your attorney, legal advisor or counsel when making decisions about your estate. **AF**

CLINICAL TRIALS

Clinical trials are conducted in four different phases with each phase serving a specific purpose to researchers.



This infographic was originally published in the Winter/Spring 2016 issue of City of Hope's City News magazine. Used with permission of City of Hope.



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continued support



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