Summer Newsletter

We're in the heat of the summer - literally in Georgia - and it is a fun time to get outdoors. Remember though that people living with ALS need to take extra care in staying shaded, keeping hydrated, and watching for breathing problems. Get out to enjoy but be prepared and mindful. Did you know we have a page on our website with patient resources and other informative articles about living with ALS? You can find it [here](https://www.als.emory.edu).

In this issue of our newsletter you will see an article by Dr. Fournier regarding the recent signing of the federal Right To Try bill. Dr. Fournier addresses the pros, cons, and the unknowns in this very informative piece.

We have exciting news for our Veterans - a new multidisciplinary ALS clinic at the Atlanta VA Medical Center! Read the details below.

Wondering how you can support us? There are three fundraising events in September that will benefit the Emory ALS Center. Choose from running/walking, cycling (various distances available), or golfing - something for everyone. We hope you'll participate in one of the events; make sure to share with your friends and family as well.

Visit our website at [www.als.emory.edu](https://www.als.emory.edu) and our [Facebook page](https://www.facebook.com/EmoryALS) to stay up to date.

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Research News

**Right-to-Try: the Pros, Cons, and Unknowns**

By Christina Fournier, MD

The federal "Right- to-Try" bill was signed into law on May 30, 2018. This legislation allows patients with a terminal illness, such as ALS, to seek out drug treatments that have successfully completed Phase 1 clinical trial safety testing, but are not yet FDA-approved, and therefore the effectiveness is unknown. Prior to passage of this legislation, unapproved drugs have only been available to terminally ill patients with FDA approval through the compassionate use program, detailed below.
From 2005-2014, 10,939 requests for expanded access of investigational drugs were submitted to the FDA, and 99% of these requests were granted. It is unknown how many patients sought investigational drugs during that time period but did not have applications filed due to lack of permission from the physician or drug company. Under the new Right-to-Try legislation, the step of seeking FDA approval is removed from the process, but the other steps remain the same, as shown below.

Under new Right-to-Try laws, drug companies must still notify the FDA when a patient is given non-approved medications, and they must also report any adverse events that occur in Right-to-Try patients. Patients must still find a physician that agrees to prescribe and manage the drug and must still get permission from the drug company to provide the therapy under these new laws.

Advocates for Right-to-Try are hopeful that by removing the need for FDA permission, the sickest patients will have faster access to experimental therapies. Supporters of the legislation note that terminal patients often do not qualify for clinical trials and will not survive long enough for new drug treatments to come to market. Right-to-Try proponents believe that although there are risks associated with unproven treatments, a patient who has exhausted standard treatment options should have the right to decide to whether or not they seek out alternatives. Critics of Right-to-Try are concerned that without sufficient FDA oversight, patients could be harmed by drugs where the patients and prescribers are unaware of drug risks. Right-to-Try opponents note that without FDA oversight, the most vulnerable patients may be taken advantage of by unethical providers seeking profit. Opponents also note that Right-to-Try could be giving false hope to patients by giving access to drugs that may not help and could be harmful.

It is unknown how much the new Right-to-Try laws will actually expand access to non-approved therapies. Experts on both sides of the issue note that the Right-to-Try law in its current form does not actually give patients the right to receive experimental therapies, only the right to ask for them, and the impact of this law may be modest. Pharmaceutical companies are expected in many cases to be reluctant to provide non-approved drugs to
patients outside of clinical trial process as complications in these sicker patients could hinder the FDA-approval of their drug and pose an unnecessary liability for the company. For patients with ALS, the impact of the new law remains uncertain.

Looking for ways to help our team?

We are looking to build our team of volunteers at our multidisciplinary clinic, which takes place two Fridays each month. Volunteers help us in a variety of ways, the best is visiting with the pALS and caregivers, offering snacks, and helping to make the appointment a little easier for them. Volunteers are scheduled in two hour shifts between 9am and 4pm. We especially need afternoon volunteers. If you are interested, contact Karen Duffy.

New Multidisciplinary ALS Clinic at the Atlanta VA Medical Center

Veterans who receive ALS care at the Atlanta VA Medical Center will now have access to services from a multidisciplinary team during VA neurology appointments. As of June 2018, the ALS clinic meets on the first and third Monday morning of each month. Patients will continue to see Dr. Fournier, but now in addition, patients will also be able to see a physical therapist, occupational therapist, speech therapist, social worker, and research coordinator in the same location without having to schedule additional appointments. ALS clinic visits with the full team are now expected to take at least 90 minutes, but should streamline ALS care and reduce the number of future in-person visits that are needed to see different specialists.

Any veteran with ALS who does currently receive VA healthcare should enroll in the VA healthcare system. For unknown reasons, military veterans are at greater risk for developing ALS compared to civilians. As a result, the Department of Veteran affairs determined that any veteran diagnosed with ALS would be automatically service connected for this diagnosis. This 100% service connection means that veterans can receive all of their healthcare, medications, and equipment needs at no cost through the VA. Service connected veterans with ALS are also eligible to receive disability compensation and receive grants for housing adaptations and vehicle allowances. Anyone who served at least 90 days of continuous active duty in the U.S. military may qualify for VA benefits.

If you are not currently enrolled in the VA healthcare system, the first step is to complete a copy of the 10-10EZ form (https://www.va.gov/vaforms/medical/pdf/1010EZ-fillable.pdf) and submit it to the Atlanta VA Eligibility Office. If you need VA assistance completing the form, you can call 1-877-222-VETS (8387). Once completed, submit this form and a copy of your DD 214 form (the Certificate of Release or Discharge from Active Duty form) to the Atlanta VA Eligibility Office. Contact information for the Atlanta VA Eligibility Office can be found here: https://www.atlanta.va.gov/patients/eligibility.asp.

We also recommend requesting assistance from your local Paralyzed Veterans of America (PVA) representative and sending him a copy of the above documents so he can help facilitate and track your claim. His name is Mitch Hinkle, email address Mitchell@pva.org, phone number 404-929-5333, fax 404-929-5337. Upon request, we at the Emory ALS Center can send copies of your recent records and a VA-specific ALS disability questionnaire form to Mitch Hinkle on your behalf to confirm the ALS diagnosis and medical eligibility for the 100% service connection.
Three September Events to Support Emory ALS Center

40th Annual Avondale Estates Labor Day Race

Monday, September 3, 2018

Run or walk the beautiful tree lined streets of Avondale Estates in this annual event that benefits the Emory ALS Center. There is a 1 mile novice race and a 5k run/walk race.

CLICK HERE to register.

Bike Ride for ALS - Van Purser Ride for the Cure

Sunday, September 16, 2018

Categories: 22 miles, 35 mile, 67 miles, 35 mile timed, 65 mile timed, 100 mile (100 mile timed) Benefiting Emory ALS Center.

CLICK HERE to register.

Volunteers are needed! Contact mydestinationtrainer@gmail.com to volunteer.

6th Annual Terance Mathis Celebrity Golf Classic

Benefiting the Emory ALS Center

September 16-17, 2018
Sunday - 9/16/18 - Topgolf Alpharetta
Monday - 9/17/18 - St. Marlo Country Club

If you are not a golfer, there is an option to attend just the Sunday evening event. There will be a guest speaker, food, drinks, silent auction, and golf games in the bays at Topgolf. CLICK HERE for a Sunday only ticket.

Monday's tournament will begin with a Helicopter Ball Drop just before the shotgun start. Anyone can purchase a golf ball for the helicopter for a chance to win $500! Lunch is provided and an awards ceremony will follow the round. Registration for the golf tournament INCLUDES ticket to Sunday's event at Topgolf. CLICK HERE to register.

You have been included in this newsletter as a service we offer at the Emory ALS Center. You will receive our quarterly newsletter, notification of upcoming conferences, symposiums, and special events. If you prefer not to be on this list, please contact Karen Duffy at kduffy2@emory.edu to be removed. No other person on the list is able to view your address and your information will not be shared.