Collaborating for a Cure

What is Collaboration for a Cure?

In 2015, the **Les Turner ALS Foundation**, along with representatives from 18 different ALS organizations across the country, met to discuss how we can work together on a variety of issues affecting all ALS groups to best accomplish our shared vision of defeating ALS. This group, called Collaboration for a Cure (C4C), is happy to report that its first area of focus has just launched: **creating a pathway for ALS drug development to improve and speed up the FDA approval process.**

Why does it take so long to development ALS drug treatments?

Drug development is a very expensive and time consuming process. In the U.S., the cost of developing a new drug can cost $2 billion and take as long as 15 years to bring to market. The process is even more complicated for a rare disease such as ALS due to the smaller number of patients available for clinical trials and the various forms of the disease. The fact that over 50 randomized, controlled trials in ALS have taken place in the last 50 years with only one treatment approved is all the evidence we need to know how important this area is for us to focus.

What is an FDA guidance and why is it necessary?

Currently, **there is no FDA guidance specific to ALS drug development**. FDA guidances are official documents that explain the agency's interpretation of a specific regulatory issue. The lack of an FDA guidance document inhibits drug development and clinical trials because the pharmaceutical industry is reluctant to take the risks to develop new therapies without the clarity of knowing how the FDA will interpret the regulatory guidelines.

C4C's goals of developing a new FDA drug guidance are to:

- Speed ALS drug development and approvals
- Provide patient input on how FDA and pharmaceutical companies should approach therapeutic development
- Inform FDA about all aspects of ALS
- Incentivize ALS therapy development

How is the guidance being developed?

The C4C drug guidance steering committee is composed of over 100 interested parties including: academic, clinical and research experts from 30 different institutions, patients, caregivers and representatives from over 10 patient advocacy organizations, industry leaders and government representatives from the National Institutes of Health (NIH) and the Centers for Disease Control (CDC). For the past year, the Guidance committee has worked tirelessly to create a **Draft ALS Drug Development Guidance.** This roadmap will provide clarification on how the FDA will review ALS candidate treatments.

Under the FDA Safety and Innovation Act of 2012, which promotes patient-focused drug development, the guidance committee has given patients and their families a critical role in the development of this document. We are encouraging patients and caregivers to let their voices be heard and help educate both the pharmaceutical industry and the FDA.

What is the timing for the guidance and when will it be approved?

The draft guidance document has just been made available for a 30-day public comment period that ends May 30, 2016 and will be submitted to the FDA in June of 2016. Once submitted, the FDA will internally review, revise and produce an official FDA guidance on ALS drug development. The guidance steering committee expects an official FDA guidance by the end of 2016.

Once the FDA publishes its version of the guidance, the ALS community will have another opportunity to provide comments. As this is a living document, the entire ALS community will continue to have input to update the guidance as new discoveries and advances in the field are made.

How will this affect clinical trials in ALS?

Clinical trial guidelines serve as “best practices” for clinical trial design and are used by researchers and the pharmaceutical industry to provide structure for the design and conduct of clinical trials in ALS. A parallel effort is underway to update the ALS clinical trial guidelines, which were originally published in 1999, with the goal of improving the number and accessibility of clinical trials in ALS.

Since both ALS draft drug development guidance and updated ALS clinical trials guidelines are being prepared at the same time, many of the same members of the ALS community are working on both initiatives so that there is consistency across both efforts.

Who is paying for it?

Funding for the legal and other costs to draft the guidance is being made by The ALS Association using monies received from the Ice Bucket Challenge. The other C4C organizations are donating their time and expertise to make this initiative a reality.

Summary:

The members of C4C believe we are stronger when we come together for a common cause. The **Les Turner ALS Foundation** is pleased to be part of this consortium of leading ALS organizations and to be able to report this new initiative to improve access to the best treatments available for ALS. This is the first of several new initiatives, so stay tuned for more information from this group in the near future.

Members of C4C:

As of May 2016, the members of Collaboration for a Cure, alongside the **Les Turner ALS Foundation**, include: ALS Canada, ALS ETF, ALS Finding a Cure, Answer ALS, ALS Hope Foundation, ALS Therapy Alliance, ALS Therapy Development Institute, ALS Worldwide, International Alliance of ALS/MND Associations, Iron Horse Foundation, Kevin Turner Foundation, Muscular Dystrophy Association, Prize4Life, PROJECT A.L.S., Target ALS, Team Gleason, The ALS Association, and The Robert Packard Center for ALS Research at Johns Hopkins.