Right to Try

Background

The desire for someone living with ALS to try experimental therapeutics that lack complete knowledge of safety and/or efficacy is understandable. To many, the nature of the disease creates a situation where the risk of benefit often outweighs the risk of getting worse or death.

However, the existence of a Right to Try legislation in any form often does not result in extensive utilization by a country's citizens because it also requires the company that owns the treatment to comply.

For Right to Try to result in someone with ALS receiving an unapproved, often experimental, treatment, the following three things must ALL be in place.

1) Some form of Right to Try legislation must exist, where government regulatory barriers that would normally safeguard citizens against unproven and unapproved treatments are reduced or removed.
    - Some countries have been willing to pass this type of legislation.
2) An owner of a treatment, often a small or large pharmaceutical company, is willing to provide the treatment, usually while still in the clinical trial process.
    - Many companies with experimental treatments will hesitate to provide it through Right to Try while it remains in clinical development as any adverse events or misuse of the treatment under non-controlled conditions could jeopardize their program and potential to bring the treatment to market. Such a situation may not only be detrimental for the company/owner, but everyone living with ALS if an effective treatment is called into question due to a Right to Try issue.
3) Cost coverage for the treatment must be identified, either through the owner providing it free of charge or the recipient paying out-of-pocket.
    - No oversight from government can allow for an owner to set their own price, which may be very high and exclusionary to some or many.
    - An owner may set prices at very high levels to compensate for risk of providing the treatment during an ongoing clinical development program.
    - Owners who don't wish to make individuals pay out-of-pocket for what are often very expensive treatments to manufacture and deliver, often won't be able to afford provision of the treatment to all through Right to Try.
    - Owners who decide to provide a limited amount of free treatment to a select group of people would likely be subject to scrutiny in a potential future market for them.

An additional, potential consequence of Right to Try legislation is the reduced level of protection against illegitimate companies/owners with products that may be unsafe or knowingly ineffective. Providing government legislated access to dubious treatments may assign a level of legitimacy to them on par with companies/owners who are taking the appropriate clinical trial steps to prove safety and efficacy of their product.

Recommendation

The SAC recommends that members of the Alliance should refrain from making any opinion-based statements about Right to Try and should refer those with questions to this document. Members should be aware of the realities of Right to Try (listed above) and compassionately provide information that balances an understanding of the position people with ALS are in, with the reasons why Right to Try

legislation is not the only step in accessing experimental or unapproved treatments. Medical advice regarding any specific treatment should be deferred to an individual's own clinician. Further objective information about what is known regarding a specific treatment in development may be sought through the SAC.

Further information

Often companies want to help people with ALS and truly care about developing and providing an effective therapy as quickly as possible. It is common for prominent clinical trials to offer extension studies, where all participants, regardless of whether they were on treatment or placebo during the trial, can receive the treatment at the company expense. These mechanisms allow for the treatment to continue in a controlled situation where risk is minimized and safety data can be collected.