

FDA Concerns and ALS WORLDWIDE Response:

1. ****Safety** – Iplex is safe for infants and young children and has already been approved for short growth stature. If not for the patent dispute, Iplex would still be available for ALS patients. FDA documents dated in 1996 by Dr. Katz and Dr. Temple agree 'Myotrophin is safe' for ALS. Further, over 150 ALS patients in Italy have used Iplex for the past 2 years with no adverse effects.
2. **Availability** – ALS patients self select. Most are on disability. Medicare will not pay for it. Cost is prohibitive. No more than 100 to 200 patients, if that, will be able to pay the cost for this drug. Insmed, the producer, is confident that requests can be filled.
3. **Interferes with Clinical Trial**- At this time, there is only discussion of a future clinical trial. Putting a clinical trial in place takes a great deal of time, money, effort and interest, none of which exist at this time. And even if it did, patients who qualify can't have had the disease for less than a year or more than 2 years because they are then too advanced. With 35,000 ALS patients in the United States and only 200 that can afford the medication, that leaves 34,800 patients for a clinical trial if and when one takes place.

***Terminology**

1. Myotrophin – IGF-1 (free growth hormone) was tested over a 15 year period for ALS patients in 3 separate clinical trials. Results are inconclusive. Myotrophin found safe for ALS but with no measureable benefits. Therefore has not been approved for ALS.
2. Increlex – IGF-1 (free growth hormone) Identical to Myotrophin, produced by a different company, FDA approved for short growth stature for infants and young children. It is available by off label cross prescription for ALS patients but provides very little symptom relief.
3. Iplex – IGF-1 and BP-3 (free growth hormone combined with binding protein) Improved version of Myotrophin. FDA approved for short growth stature for infants and young children. Anecdotal evidence that it reduces ALS symptoms and improves quality of life for patients.
4. IND – Investigational New Drug – Status given to any drug that has not been approved by the FDA. Iplex actually has been approved but was removed from the market because of the patent dispute.
5. IRB – Independent Review Board – Often connected to a University or Hospital. But for many physicians, they have no such connections. For scientists and physicians conducting clinical trials, there are companies who provide services that are required by the FDA and charge approximately \$2000 per patient. For ALS patients whose doctor is not affiliated with such a company, they must pay an IRB the money themselves for this oversight just to be able to qualify. In truth, the IRB will do little more than collect the money because no trial is actually taking place.

Why is Iplex better than Increlex for ALS?

Free IGF-1 travels throughout the body to all the muscle groups and bones, everywhere which is a good thing for short growth stature because the entire body needs it. But in ALS, the symptoms appear in various places in various degrees. Iplex is 'intelligent' IGF-1 because it goes where it is most needed.