

## Andalusian Initiative for Advanced Therapies



**Preclinical  
regulatory  
advice**



**25 clinical trials** with  
advanced therapies

### THERAPEUTIC ÁREA / DISEASE

Critical Limb Ischemia
Acute Myocardial Infarction
Dilated Cardiomyopathy
Chronic Ischemic Cardiopathy
Stroke
Extended Liver Resection
Multiple Sclerosis
Amiotrophic Lateral Sclerosis
Critical Limb Ischemia
Faecal Incontinence
Faecal Incontinence
Chronic GVHD
HIV Infect.- Disc Im. Response
Critical Limb Ischemia
Multiple Sclerosis
Corneal Ulcers
Tumors, Burns, Ulcers

**Clinical  
protocol**

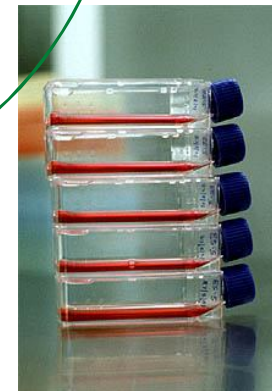
**Support the  
development of  
clinical research  
with ATMPs**

**GMP facility**

network of 10 GMP laboratories

manufacturing 12 authorized  
advanced therapies  
-MSC, artificial skin, artificial  
cornea, MNC..

**Investigational  
medicinal  
product**



[www.juntadeandalucia.es/terapiasavanzadas](http://www.juntadeandalucia.es/terapiasavanzadas)

# Clinical trial in ALS

Clinical trial **phase I/II**, multicentric, randomized, controlled with placebo, triple blind.

**N = 40 patients with probable or defined ALS** by El Escorial criteria -**28** included from July 2014

**IND = Autologous expanded mesenchymal stem cells from fat tissue**

**Groups: 0, 1, 2, 4 M cells/kg of body weight iv –single infusion**

**Follow-up:** 6 months (+ 36 months)

## 1ary OUTCOME Safety

- Adverse effects related to IND and procedure
- Reactions in the infusion site
- New neurological deficits inconsistent with disease progression

## 2ary OUTCOME -Efficacy

- **Clinical** variables –ALSFRS-R, MMT, FVC%, time to gastrostomy, tracheostomy, survival
- **Immunological** –Tregs, TLRs in CD4+ & CD8+, IL17 production, in vitro assays of functional capacity of Tregs
- **Neurophysiology** - MUNE, amplitude and stimulation threshold in motor evoked potentials
- **MR** quantification of muscle mass in upper and lower extremities