

2023

International Patient Advocacy Group Meeting



ALAME

Coordination Across Countries, Groups and Differences

17 Advocacy Groups 15 Countries



- Paraguay
- Chile
- Uruguay
- Argentina
- Peru
- Ecuador
- Costa Rica
- República Dominicana
- Colombia
- Brazil
- Mexico
- Venezuela
- Cuba
- Panamá
- Bolivia

ALAME was born in 2020

Main Objectives:

- Make visible the disease in our countries.
- Achieve empathy of our communities. LA is going through a rough economic & government situation, and rare diseases are not a priority.
- Know each country needs, what do we have in common, what our differences are? How can we help each other?

ALAME

Our Needs



5500 Registered Patients

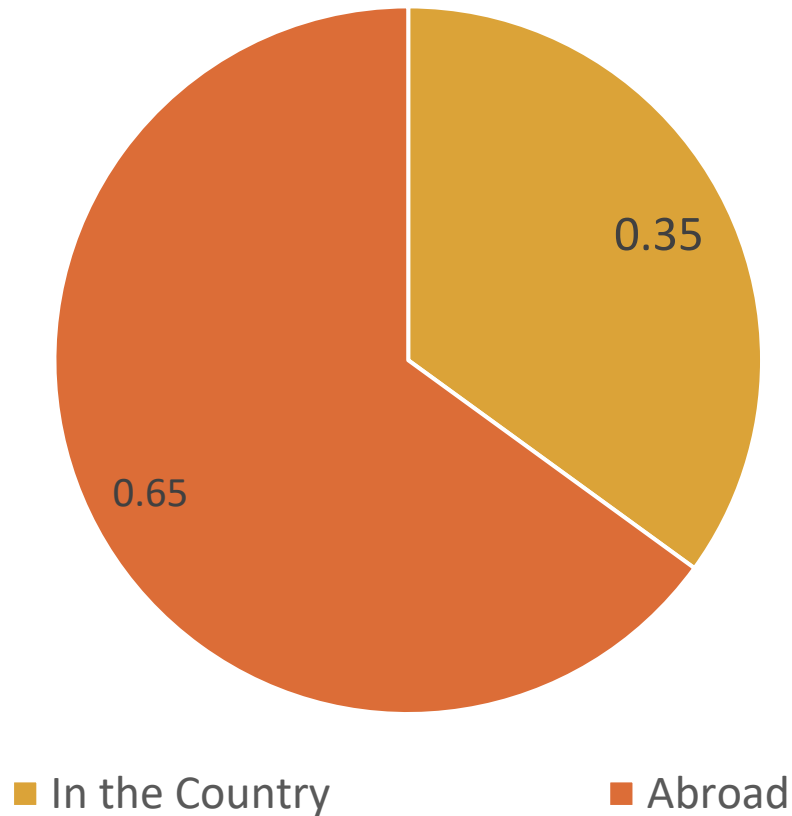
- 29% SMA Type I
- 49% SMA Type II
- 19% SMA Type III
- 2% SMA Type IV
- 1% Others

All countries have their own registry, only a few working with a formal CRM



Access to Diagnose

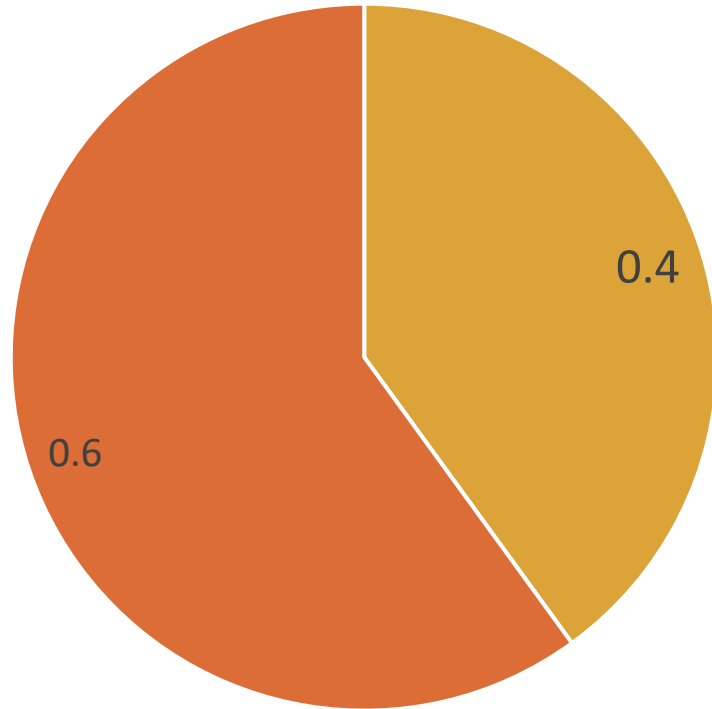
Molecular Tests



- Only 35% of the countries - 5 of them - perform molecular studies (and SMN2) in their territory.
- Only 2 of the 9 countries that don't have access, receive help from lab programmes.

Standards of Care

Treatment Centers



- Countries with Multidisciplinary TC
- Countries that don't have MTC

- 60% of LA countries, still do not have multidisciplinary treatment centers to recommend patients when they receive diagnose.
- Only 3 countries have guides for Standards of Care certified by Ministry of Health
















































1800 Patients in Treatment

Access to Treatment



In 2023, more than 65% of SMA Latin American Population do not have Access to Treatment at all

	SPINRAZA	ZOLGENSMA	EVRYSDI	
Argentina				Paid by government with restrictions. Evrysdi recently registered, still no public coverage.
Bolivia				
Brazil				Paid by government with restrictions. Evrysdi recently registered, still no public coverage.
Chile				Those depending on public health, access through amparo trials. Others have access through private insurance.
Colombia				Evrysdi in registration process.
Costa Rica				Access through amparo trials.
Cuba				
Ecuador				Access through amparo trials.
México				Access through amparo trials/ clinical trials & private insurance. Treatments are not covered by public health.
Panamá				Only Compassionate use of Evrysdi
Paraguay				Public Health provide Evrysdi, others have access through amparo trials.
Peru				Access through compassionate use & MH.
República Dominicana				
Uruguay				
Venezuela				

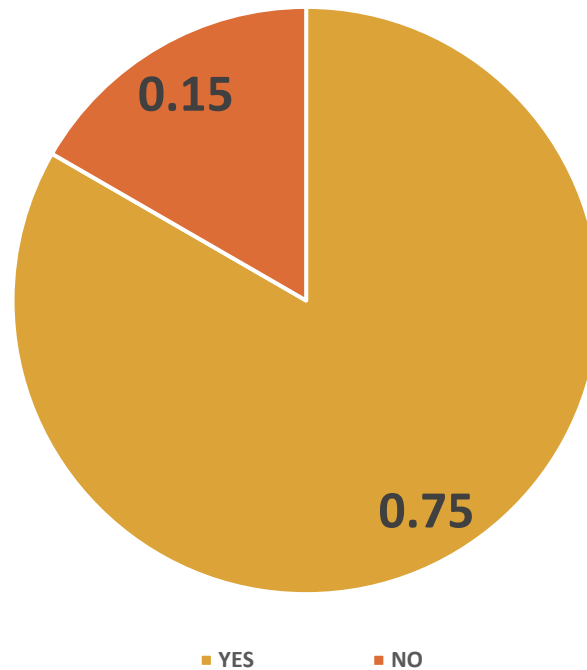
Access to Treatment & Clinical Trials

- More than 50% of the actual treated population have had access through amparo trials.
- More than 70% of Latin American countries had their first dosed patient after 2020.
- Chile, Mexico, Colombia, Brazil & Argentina had participated in different clinical trials, almost 70% of the countries have been left out this possibility.



Research &
Clinical Trial
opportunities

Newborn Screening



Even 75% of LA countries have NBS Law, there are 0 countries with SMA included in them.

- Brazil is adding 53 detectable diseases, and will include SMA in one of the stages. Three programs are already being carried out in different cities.
- Argentina has a pilot project starting in September with the National Medicine Academy.

ALAME has been working on:

Strengthen
as alliance

- Representing LA patients in meetings with Roche, Biogen & Novartis, with focus in delivering possibilities of diagnose in every country, working together for treatment registrations in every country, and improving access to treatments.
- Made several surveys to understand each country needs.
- Educational programmes for Advocacy leaders from 2021 on (with focus on the needs presented by each country).
- Participated in SOLANE and Treat NMD and SMA Europe meetings.
- Working with SMA Europe, to learn from their experience.
- Media LA campaign through Awareness month.
- Kids and Youngs virtual meetings with participation from all the countries.

