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# Bring good treatment for cystinosis patients around the globe

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Approximately 60 patients

Spanish Cystinosis Association (ACE) was founded in 2016



# Patient care

Patient care and care management is heterogenous. There isn't a single care-model.

Care is centered on kidneys and eyes

Val d'Hebron Hospital in Barcelona is a nationwide reference center

## Access to treatment

All patients have access to Cistagon and formula eye drops prepared in hospitals.

In February 2019, the government published a resolution against making Procysbi available. Only 9 patients have access to it.

In October 2018, the government published a resolution against making Cystadrops available. Only 14 patients have access to it.

These treatments may be requested for individual patients. If approved, the cost must be covered by their hospital.

Only half of the treatments approved by the EMA are approved in Spain. The average waiting time for approval is 15 months.

# Treatment monitoring

Only one center tests white blood cell cystine levels. Blood samples from all the country are sent there.

Results are sometimes unreliable and fluctuating.

Adjusting cysteamine treatment based on these results could be dangerous.

# Hopes for the future

Access for all patients to all treatments approved by the EMA.

Collaboration and knowledge-sharing among medical teams, to provide the best care wherever the patients are.

Participation of adults with cystinosis in conferences and in the association.

**Thank you for your attention**