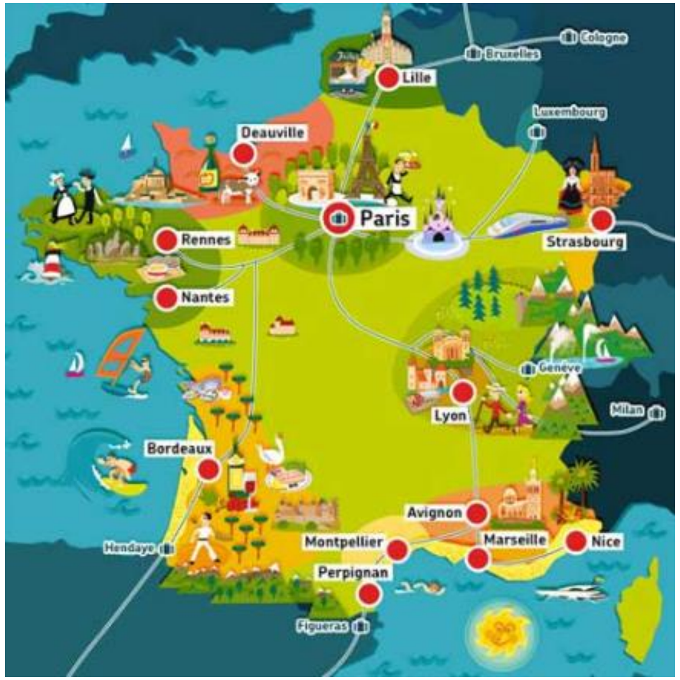
Cystinosis Network Europe



Bring good treatment for cystinosis patients in France

Aurélia Bertholet-Thomas – Pediatric nephrologist - Lyon

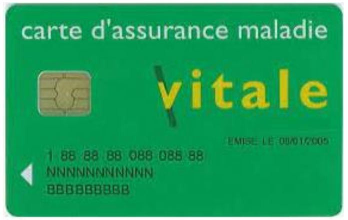
Aude Servais – Adult nephrologist –Necker- Paris

ORKID FILIÈRE ORPHAN KIDNEY DISEASES

maladies rares

european society for paediatric nephrology

Are you able to give the optimum treatment to cystinosis patients in France?



- Yes!
- One of the best Public heath care system in the world
- No private complementary health insurance needed
- Treatment available and reimbursed in full:
  - All cysteamine treatment: Procysbi®/Cystagon®/Cystadrops®
  - Growth Hormon
  - Immunosuppressive treatment
  - Extrarenal diseases as hypothyroïdism, diabete
- Electrolytic supplementation for patients with native kidneys: sometimes not reimbursed in full

In what sense do you think treatment of cystinosis in France differs from other countries?

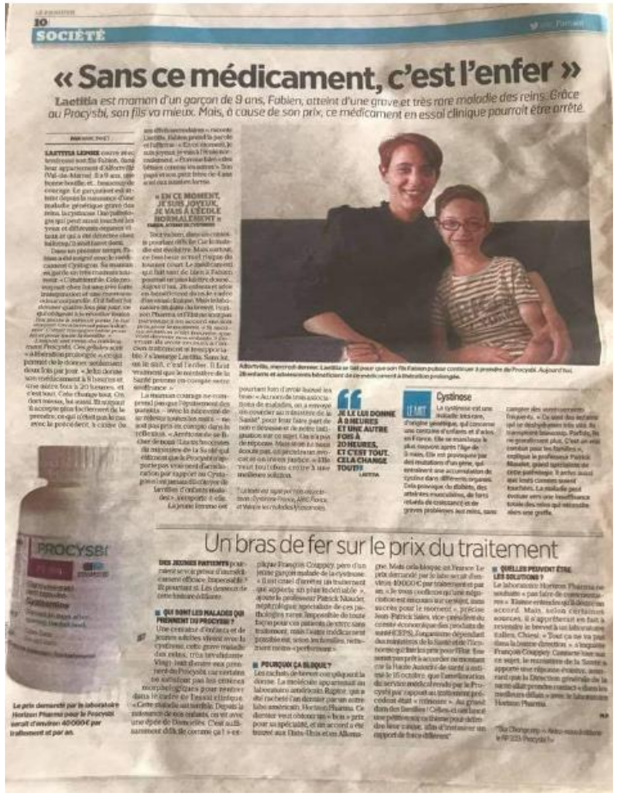
- Available and reimbursed in full

⟶ Long and hard price negociation!

Procysbi®: Marketing authorisation 09/2013

Patient availability: 06/2018

- Freedom of medical prescription
  - No financial authority
  - « Off-label » drug use based on consensus expert, litterature
- Early access authorization of new treatment in rare disease



10

SOCIÉTÉ

« Sans ce médicament, c'est l'enfer »

Laetitia est maman d'un garçon de 9 ans, Fabien, atteint d'une grave et très rare maladie des reins. Grâce au Procysbi, son fils va mieux. Mais, à cause de son prix, ce médicament en essai clinique pourrait être arrêté.

« JE LE LUI DONNE À 8 HEURES ET UNE AUTRE FOIS À 20 HEURES, ET C'EST TOUT. CELA CHANGE TOUT! »

Cystinose

Un bras de fer sur le prix du traitement

« Without this treatment, this is hell »

Newspaper « Le Parisien » 30/05/2017

What do you think about the availability of medicines and care to **all** cystinosis patients in France?

- Good availability
- 2 places of treatment delivery
  - Procysbi®/Cystagon®: hospital delivery only, far from home
  - Cystadrops®: drugstore delivery

→ Limitation of compliance

- Good health care organisation: pediatric/adult medical experts
  - Rare diseases centers
  - Rare diases Network
  - Patients can travel to the center of their choice
  - French Protocol

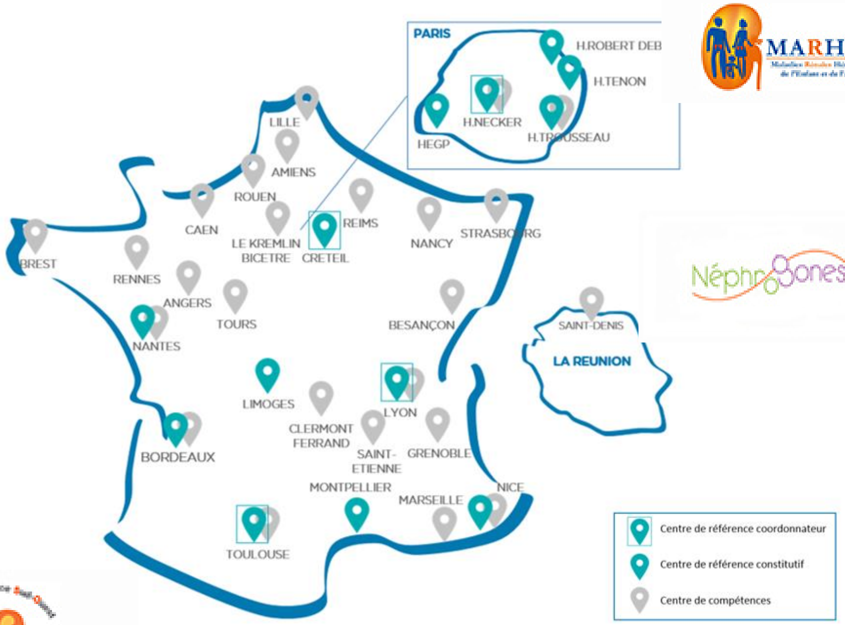
→ However knowledge about cystinosis is limited in adults departments outside of the reference centers

ORKiD FILIÈRE ORPHAN KIDNEY DISEASES

MARHEA

NéphroGones

SO RARE



How would you describe the transition from paediatric to adult care in France?

- Which one? Transition as “a transplanted patient” or “a cystinosis patient”
  - Transplanted patient: transition protocol in all centers
  - Cystinosis:
    - Only 2 centers: Necker hospital – Paris and Lyon
    - Dedicated transition program, multidisciplinary consultations
- Difficulties to identify adult nephrologists involved in cystinosis in all centers: limited expertise in adult units
- Joint transition visit: paediatrician and adult nephrologist
  - less patients lost to follow up
- Problems of adhesion to treatment during that period : role of education nurses?

What improvements in cystinosis treatment are required in France?
What do expect for the future?

- More suitable packaging depending on the patient age:

Procysbi® 75 mg: 250 capsules/bottle – can be used 1 month after the first opening

A 5 yrs old girl: 6 pills/d of 75mg capsules of Procysbi = 180/months + 130 capsules (1300 euros) in the trash/month…

Adult patients: 20 capsules per day!

- Capsule blue pigment: long-term impact?

→ New packaging of cysteamine

Less expensive

Emerging approaches: when will hematopoietic stem cell transplantation be available?