Servizio Sanitario Regionale
Dipartimento Tutela della Salute e Politiche Sanitarie

Azienda Ospedaliero Universitaria "Renato Dulbecco" di Catanzaro

UMG Dubium sapientiae initium

THE DISEASE IS RARE
THE SUFFERING IS FOR EVERYONE

A CASE REPORT

MD NATURALE 1 , **A. BRESCIA** 2 , S. ESPOSITO 2 , M. ZITO 2 , C. MONOPOLI 2 , MG ALCARO 2 , D. CASUSCELLI 2 , M. DE FINA 2 , GM MARRAZZO 2 , B. SPINOSO 2 , **A.E. DE FRANCESCO** 2.

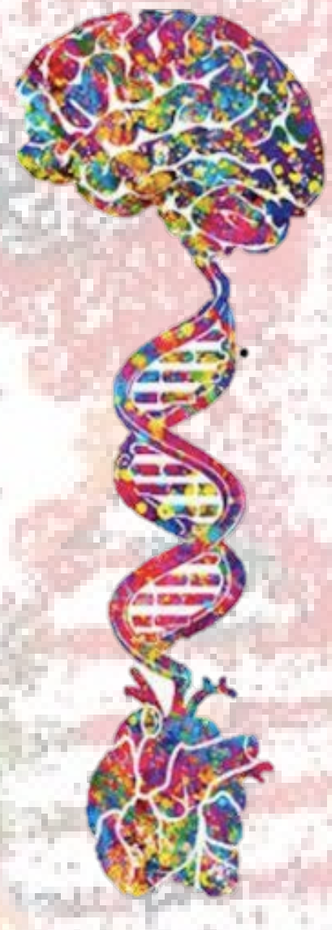
1 MAGNA GRAECIA UNIVERSITY, DOCTORATE IN LIFE SCIENCES, CATANZARO, ITALY.
2 AOU RENATO DULBECCO-PO MATER DOMINI, PHARMACY, CATANZARO, ITALY.

CLN2 disease (Neuronal Ceridolipofuscinosis Type 2) is an ultra-rare neurodegenerative lysosomal storage disease caused by an enzyme deficiency of tripeptidyl peptidase 1.

Early diagnosis and treatment of CLN2 disease are therefore crucial to preserve function and slow decline for as long as possible.

The patient is a girl born in 2005. A personalized pharmacological protocol was created, with continuous monitoring of vital signs, before, during and after drug administration.

The Clinical Pharmacy Laboratory guarantees sterility and asepticity in the preparation of the drug



THE DRUG IS STORED AT -20°C. THE STANDARD REGIMEN INVOLVES A DOSE OF 300 MG, EVERY 2 WEEKS

TREATMENT AND PREPARATION ARE CARRIED OUT IN THE CLINICAL PHARMACY LABORATORY.

THE CHEMICAL CHARACTERISTICS OF THE FINISHED PRODUCT HAVE BEEN VERIFIED

IT IS DELIVERED TO THE DEPARTMENT FOR SUBSEQUENT ADMINISTRATION

TEMPORARY SUSPENSIONS DUE TO INFECTIONS

The ICV delivery system carries potential risks, such as infections, which may require antibiotic treatment or device replacement.

4CPS-289: SCAN ME!

Patient appear to experience a slower worsening of symptoms.

This confirms the importance of encouraging and supporting clinical research, especially in rare diseases, which are often difficult to recognize and treat.