

INTERNATIONAL WORKSHOP ON GENE THERAPY IN RARE DISEASES

October 23rd–24 th / 2025

HOSPITAL UNIVERSITARIO QUIRÓNSALUD. CLASSROOM / AVDA. GÓMEZ LAGUNA 159 / ZARAGOZA. SPAIN

THURSDAY, 23rd OCTOBER 2025

-  16:00 – 16:15 | Welcome and Introduction. Ramón Boria - General Director Health Care. Government of Aragón. Miguel Eguizabal Eguizabal - Medical Director. HUQS. Zaragoza.
-  16:15 – 16:20 | Workshop Agenda & Objectives. Prof. Pilar Giraldo - Hematology. GEEDL Zaragoza.
-  16:20 – 16:45 | Historical Perspective of Treatment in Lysosomal Diseases. Prof. Gregory Pastores - University College Dublin. Ireland
-  16:45 – 17:15 | Experience in Gene Therapy for Lysosomal Diseases (Fabry & Gaucher). Prof. Derralyn Hughes - Royal Free Hospital. UCL. NHS Foundation Trust. London. UK
-  17:15 – 17:45 | Local Experience in Gaucher Disease Gene Therapy. Dra. Diana Heredia/Prof. Pilar Giraldo - Pharmacy Dpt/Hematology. HUQS. Zaragoza.
-  17:45 – 18:15 | Genetic confirmation as an essential criterion for the implementation of advanced therapies. Prof. Belén Pérez - Centro de Diagnóstico de Enfermedades Moleculares. Universidad Autónoma de Madrid.
- 18:15 – 18:30 | Discussion & Conclusions

FRIDAY, 24th OCTOBER 2025

-  16:00 – 16:10 | Agenda & Objectives of the Day. Moderator Dr. José Mª Grasa - Hematology. HUQS. Zaragoza.
-  16:10 – 16:45 | Gene Therapy Results in Congenital Anemias. Dr. Julián Sevilla Navarro - Hematology Hospital Universitario Infantil Niño Jesús. Madrid.
-  16:45 – 17:15 | Fundamentals of Gene Therapy in Rare Diseases. The example of Pompe disease. Prof. Jordi Díaz-Manera - J.Walton Muscular Dystrophy Research Centre. Newcastle University. UK
-  17:15 – 17:45 | Customized RNA modifying molecules as potential therapeutic weapons in lysosomal diseases. Dra. Andrea Dardis. Vice-Chair IWGGD. Centro Regional de Enfermedades Raras. Hospital U. Santa Maria de la Misericordia. Udine. Italy
-  17:45 – 18:15 | New Expectations of Gene Therapy in Hemophilia. Dra. Teresa Alvarez - Hematology. Hospital Universitario La Paz. Madrid.
-  18:15 – 18:45 | Genetics and Cell Therapy : A Strategic Alliance for the Regenerative Medicine of the Future. Prof. Mª Pilar Ribate - Universidad San Jorge. Zaragoza.
- 18:45 – 19:00 | Final Discussion & Workshop Closing

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