

Histiocytosis in Argentina Since 1991

AIM

- To present the experience of a multidisciplinary study group focused on rare and complex histiocytic disorders.

Langerhans Cell Histiocytosis (LCH)

- **Disregulation / abnormal activation of Langerhans cells**
- **Involvement of skin, lymph nodes, bones, liver, lungs, spleen, Hematologic system, CNS, endocrine glands**
- **Unifocal or multifocal. Unisystemic or multisystemic. Reversible with no treatment, response to treatment or progressive fatal regardless treatment**
- **Sequelae: Bone, endocrinologic, CNS, liver, lungs**

Hemophagocytic Lymphohistiocytosis (HLH)

- **Disregulation and abnormal activation of Macrophages. Fever. Involvement of hematologic system, liver, spleen, CNS, biochemical abnormalities**
- **Bone Marrow Transplantation mandatory for HLH patients with genetic or progressive disease after initial treatment**
- **CNS sequelae**

LCH: Incidence in Argentina Hospital Garrahan

- **Mean annual rate for children < 15 years old:
5.1 / 1,000,000**
- **Hospital Nacional de Pediatría Garrahan (1987-2007):
320 patients**

Argentina Study Group

- **Jorge Braier, Diego Rosso, Guillermo Chantada, Julio Goldberg, Antonio Latella, Blanca Balancini, Patricia Bernáldez, Amelia Laterza and Matías Oleastro. Hematology/Oncology, Pathology, Pediatrics, Radiology, ENT, Dermatology and Immunology Departments. Hospital de Pediatría Garrahan.**
- **Guadalupe Rey and Daniel Pollono. Hematology/Oncology Departments. Hospitales Pediátricos Gutiérrez y Ludovica. Buenos Aires and La Plata. Argentina.**
- **Other pediatric and General Hospitals: Buenos Aires, Rosario, Cordoba and Neuquen.**

Study Group Meetings (monthly)

Hospitals Garrahan, Gutiérrez and Ludovica:

- **Discussion of clinical cases**
- **Analysis of the information of patients included in the Histiocyte Society (HS) protocols**
- **Discussion related to presentations in HS or other national / international meetings**
- **Analysis and discussion of manuscripts going to national / international journals**
- **Organization of Meetings (National / International)**

HS Clinical Research protocols

- **1991-2008: Argentina contributed to HS Protocols LCH I (1995), LCH II (1996-2000), LCH III (2001-2007)**
- **LCH S (1995-1997) (1998-2005)**
- **HLH 1994 and 2004**

- **GPOH (Austria, Germany, Netherlands, Switzerland), France, UK, Italy, Sweden, Spain, USA, Canada and Argentina**

- **Argentine Patients: LCH I: 6 - LCH II: 63 - LCH III: 118 - LCH S: 25 - HLH 94 / HLH 2004: 11**

Argentine Contribution to HS meetings (1992-2007)

- **49 abstracts of our study group in HS annual meetings (6 in collaboration with USA and Europe)**
- **Nesbit Prize (Clinical Science) given to our presentation “Reactivations in Langerhans Cell Histiocytosis” - HS meeting in Portugal (2002)**

Publications

Main Subjects:

- **Retrospective Evaluation of LCH patients at Hospital Garrahan**
- **Sclerosing Cholangitis and Liver Transplantation in LCH**
- **Pulmonary involvement in LCH - Prognosis**
- **Cytokines in LCH: IL-2r, IL-1Ra, TNF-alpha, Osteoprotegerine (Karolinska Institut, Sweden)**
- **Reactivations in LCH**
- **Progressive LCH, Late Effects, Diabetes Insipidus, LCH II study (in collaboration with HS members: USA, Canada and Europe)**

Publications

12 publications in:

- *Pediatric Hematology and Oncology*
- *Medical and Pediatric Oncology*
- *British Journal of Hematology*
- *Journal of Pediatric Hematology and Oncology*
- *Pediatric Blood and Cancer*
- *Pediatric Research*
- *Blood*

HS Committees and Study Groups: Argentina

- **Education Committee**
- **Board**
- **Protocol Study Groups: LCH II, LCH III, LCH IV, LCH S, HLH 1994, HLH 2004**
- **Late Effects Study Group**

Education. Consultations

- Lectures in Pediatric Hospitals Garrahan, Gutiérrez, Elizalde and at interhospital meetings
- Meetings in pediatric hospitals in Tucumán, Mendoza, and Mar del Plata
- Lectures in Brazil, Colombia, Mexico, and US (Dallas, Iowa, New York)

- Consultations from: Argentina, Chile, Uruguay, Brazil, Peru, Ecuador, Colombia, Venezuela, Mexico, US, Canada, Spain, UK, Israel, Australia

International Meetings in Argentina

- **International Symposia (organized by Hospital Garrahan, Histiocyte Society, Garrahan Foundation, and Argentine Society of Hematology (1995, 2004))**
- **HS Annual Meeting, Histiocyte Society, Histiocytosis Study Group of Argentina, Garrahan Foundation, Buenos Aires, 2006**
- **(Online Cure4Kids program), St Jude Hospital (USA), Hospital Garrahan, hospitals of Latin America and Spain. Monthly presentation of LCH/HLH cases. Discussion of treatment options.**

Parent Associations

- *Fundación Calloni*
- *Asociación de Histiocitosis*
- *Asociación Argentina de Histiocitosis*
- *Fundación Geiser (Rare diseases)*

The Future (1)

- **To continue with our work as previously described**
- **To improve our database**
- **To increase education on Histiocytic disorders in Argentina and, if possible, in Latin America**
- **To develop prospective studies in late effects and epidemiology**
- **To improve our knowledge of Histiocytosis in adults**

The Future (2)

- **To collaborate with national or international centers in basic research**
- **To develop, in some group of patients, national or Latin American clinical research protocols**
- **To collaborate with national (Fundación Geiser) and international rare disease organizations**
- **VERY IMPORTANT LIMITATION: NO ECONOMIC SUPPORT (ORPHAN DISEASES)**