Around 1.000 delegates participate in the 17th International Symposium on Amyloidosis, organised by the International Society of Amyloidosis and the PETHEMA Foundation, under the clinical leadership of the Hospital Clínic de Barcelona

Heart involvement at the time of diagnosis, the primary adverse prognosis factor for amyloidosis

- **Amyloidosis is a group of diseases characterised by the build-up of amyloid proteins -an apparently amorphous fibril substance- in different tissues and organs**
- **Amyloidosis is diagnosed by histology and depends on finding evidence of amyloid deposits in tissues**
- **Choice treatment for light-chain (AL) amyloidosis among patients under 65 years old in a good state of general health and with normal heart function involves chemotherapy with high dose melphalan and a salvage therapy with autologous haematopoietic stem cell transplantation**
- **There are already a range of treatments for transthyretin-related amyloidosis (TTR), with diagnoses increasing due to genetic advances and imaging techniques**
- **The scientific program involves 8 plenary sessions, 6 sessions for presenting selected "abstracts" and 5 industry-sponsored symposia, which will cover current issues**

Barcelona, 14th September 2020. Amyloidosis is a group of diseases caused by the build-up of amyloid proteins -an apparently amorphous fibril substance- in different tissues and organs. There are different types of amyloidosis, differentiated by the protein precursor of the amyloid substance, although they can be classified by aetiology (hereditary or acquired) and the distribution of amyloid deposits (localised or systemic). At least 25 proteins are capable of forming amyloid fibrils. On being a multi-organ disease, adopting the correct approach requires using multidisciplinary teams. Amyloid light-chain (AL) amyloidosis is caused by immunoglobulin light chains produced in bone marrow. With an incidence of 0.9 new cases per 100,000 population each year, it is the most common form of amyloidosis. Patients are treated by haematologists, given that it falls under the field of monoclonal gammopathies, as does multiple myeloma.

With the aim of analysing the new approaches and treatments being used to tackle amyloidosis, the International Society of Amyloidosis (ISA) and the Program for the Study and Treatment of Haematological Malignancies (PETHEMA) of the Spanish Society of Haematology and Haemotherapy (SEHH), are organising the virtual 17th International Symposium of Amyloidosis, which will be held from today through to September 18th. Dr Joan Bladé, haematologist, Hospital Clínic de Barcelona, Amyloidosis and Myeloma Service, and a world opinion leader in treating these diseases, announced during the official press conference that "there will be around 1.000 delegates from around the world

participating. This includes haematologists, nephrologists, neurologists, hepatologists, internal medicine specialists and cardiologists, all involved in tackling this multi-systemic disease". He also referred to the clinical leadership of Hospital Clínic and the PETHEMA Foundation in organising this international gathering.

In general, amyloidosis is diagnosed "by histology and depends on finding evidence of amyloid deposits in tissues", indicated the opinion leader. Fine needle aspiration biopsy of abdominal fat "is the most sensitive technique (80% sensitivity), meaning it is considered the procedure of choice". After finding evidence of amyloid, the next step would involve identifying the type of protein precursor in order to establish the definitive amyloidosis type diagnosis. The rectum, or oral or lingual mucosa, are other locations in which the presence of amyloid can be found with a biopsy. A kidney, liver, or even an endomyocardial biopsy is required to establish the diagnosis in certain situations. "The primary adverse prognosis factor is heart involvement at the time of diagnosis", he indicates.

New treatment approaches

Dr Bladé stated that this symposium "will see the analysis of the new approaches and treatments being used to tackle AL amyloidosis: combinations based on bortezomib (a proteasome inhibitor drug), alkylating agents (a class of drug used for chemotherapy), monoclonal antibodies and autologous haematopoietic stem cell transplantation". At present, "the treatment of choice for AL amyloidosis among patients under 65 years old, in a good state of general health and with normal heart function, is chemotherapy with high dose melphalan and a salvage therapy with autologous haematopoietic stem cell transplantation", he explains. "In patients achieving complete response survival may last for over 10 years", he adds. "For other patients treatment may be followed by low-dose alkylating agent-based chemotherapy and corticosteroids (melphalan and dexamethasone), along with frequent controls by haematologists, although recent years have seen the use of new treatment regimens that will allow improved results".

With respect to transthyretin-related amyloidosis (TTR), this can be hereditary due to TTR mutations, with neurological and/or heart manifestations, or derived from TTR ageing, the so-called senile cardiac amyloidosis. "There are already treatments for this type of amyloidosis, which is being diagnosed earlier and in greater numbers due to genetic advancements and the availability of more sophisticated imaging techniques", point out the opinion leader. The symposium will present results from new therapies under investigation, such as tafamidis, inotersen, or patisiran, all drugs with novel mechanisms of action targetted to reduce amyloidogenic protein production, interfere with amyloid formation, and favour its degradation, with the ultimate aim of enabling recovery of involved organs.

The scientific program of the 17th International Society of Amyloidosis Symposium is looking at AL amyloidosis and TTR-related amyloidosis in equal measure. This is borne out by the fact that each disease will be examined in a separate final session to deal with the hot topics. There will also be space to tackle other forms of the disease, however. Additionally, the program includes 8 plenary sessions, 6 sessions for presenting selected "abstracts", and 5 industry-sponsored symposia, which will cover topics such as the formation, deposition, and clearance of amyloid fibrils, the diagnosis and management of AL amyloidosis in 2020, the role of autologous haematopoietic stem cell transplantation in AL amyloidosis, the treatment of patients with AL amyloidosis not eligible for autologous transplantation, genetics and basic science regarding TTR-related amyloidosis, the clinical characteristics and monitoring of hereditary TTR-related amyloidosis, organ transplantation with systemic amyloidosis, or AA amyloidosis and other forms of the disease. Dr Per Westermark (Uppsala, Sweden) will give the opening conference, titled "Amyloidosis: classification and epidemiology". Dr Giampaolo Merlini (Pavia, Italy) will follow with a conference on "Searching for the cure for amyloid disease".

“This is undoubtedly a thorough symposium that will analyse every aspect relating to amyloidosis and will include every new advance for diagnosis, prognosis, and treatments in particular”, concludes Dr Bladé.

PETHEMA: over 4,400 patients treated in the past decade

Dr José Francisco Tomás, patron of the PETHEMA Foundation, highlighted some of the data from the SEHH cooperative group with the greatest scientific output. “During the last decade, over 4,400 patients have benefited from treatments as part of the PETHEMA clinical trials, with over one hundred hospitals in Spain and 6 in Portugal participating (and continuing to participate) in studies run by the group”, he indicates. Furthermore, “over 225 international publications have been generated in the past 5 years, with an average annual impact factor of 307.7”. All this resulting from the work carried out by PETHEMA’s independent, academic investigation subgroups in multiple myeloma, acute lymphoblastic leukaemia, acute myeloblastic leukaemia, chronic lymphocytic leukaemia, myelodysplastic syndromes, bone marrow failure, and immune thrombocytopenic purpura. “We are probably in one of the cooperative group’s best periods, and for Spanish haematology in general for that matter. This is why we are very proud to join Hospital Clínic and the International Society of Amyloidosis in leading the organisation of this international symposium”.

Dr Tomás states that “the major efforts undertaken in recent years has put Spain, and the PETHEMA group in particular, as one of the leading countries in clinical investigation in both Europe and across the world. Through the multidisciplinary and multi-centre work, and the gathering of different points of view about haematological disorders, we have managed to advance knowledge and motivate the participation of a large majority of Spanish hospitals. Health institutions need to foster and increase the incentives for clinical investigation, providing suitable funding and enabling the participation of different centres in cooperative studies. Red tape also needs to reduce to a minimum as it often slows down progression on studies”.

About the Spanish Program for the study of Haematological Treatments (PETHEMA)

PETHEMA is an independent and academic investigation cooperative group that belongs to SEHH. It comprises clinical haematologists and laboratory researchers from the Spanish public health system hospital network, some private centres, and several European and South American hospitals. Their only interest involves driving medical investigation projects in the field of haematology and haemotherapy with the aim of generating new knowledge about blood diseases, along with suitable procedures for their diagnosis, with a particular focus on treatments to cure people, or at least extend their lives without having to give up certain quality standards.

The PETHEMA Foundation itself was created with the aim of recruiting resources for the investigation activities undertaken by the different subgroups comprising PETHEMA and to provide everything required to carry out this investigational work. As such, it is managed by healthcare professionals and foundation specialists comprising a team of people with extensive investigation experience.

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