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THE IMPORTANCE OF PHARMACY DEPARTMENT CLINICAL TRIALS UNIT INTERVENTION IN A REFERENCE CENTER FOR THE TREATMENT OF PARAMYLOIDOSIS

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BACKGROUND AND IMPORTANCE

The Local Health Unit of Santo António (ULSSA), as a reference center for the treatment of familial paramyloidosis, receives patients from across the country [1]. Due to the endemic nature of this disease, the emergence of new therapeutic options is essential to ensure treatment and reduce the impact the disease has on individuals and families.

The Pharmacy Department Clinical Trials Unit (PDCTU) at ULSSA plays a crucial role, along with all stakeholders involved in clinical research, in creating evidence to ensure the efficacy and safety of new treatments.

AIM AND OBJECTIVES

Describing the activity of the PDCTU in a reference center for the investigation and treatment of Hereditary Transthyretin Amyloidosis (hATTR), between 2006 and 2023.

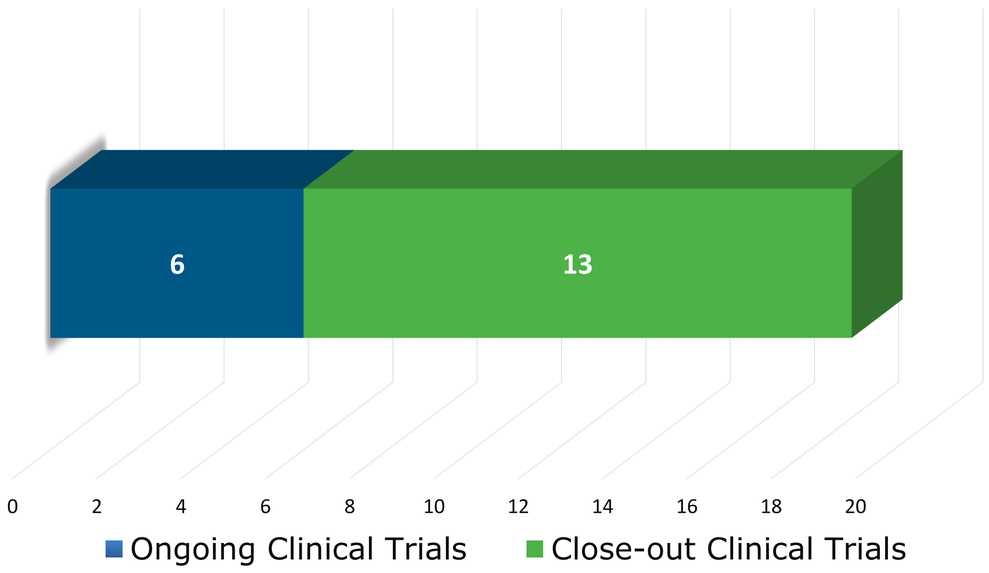
MATERIAL AND METHODS

Retrospective analysis of the participation of the PDCTU of our hospital in the clinical investigation of hATTR. For this analysis, the number of Clinical Trials (CT) started each year, the number of ongoing CT and the number of patients included in CT associated with hATTR were evaluated.

RESULTS

Since 2006, our PDCTU has participated in 21 CT. It has made a significant contribution to the approval of emerging therapies, some of which have already been granted Marketing Authorization, as is the case of Tafamidis, Inotersen and Patisiran.

NUMBER OF CLINICAL TRIALS ASSOCIATED WITH HATTR - ULSSA

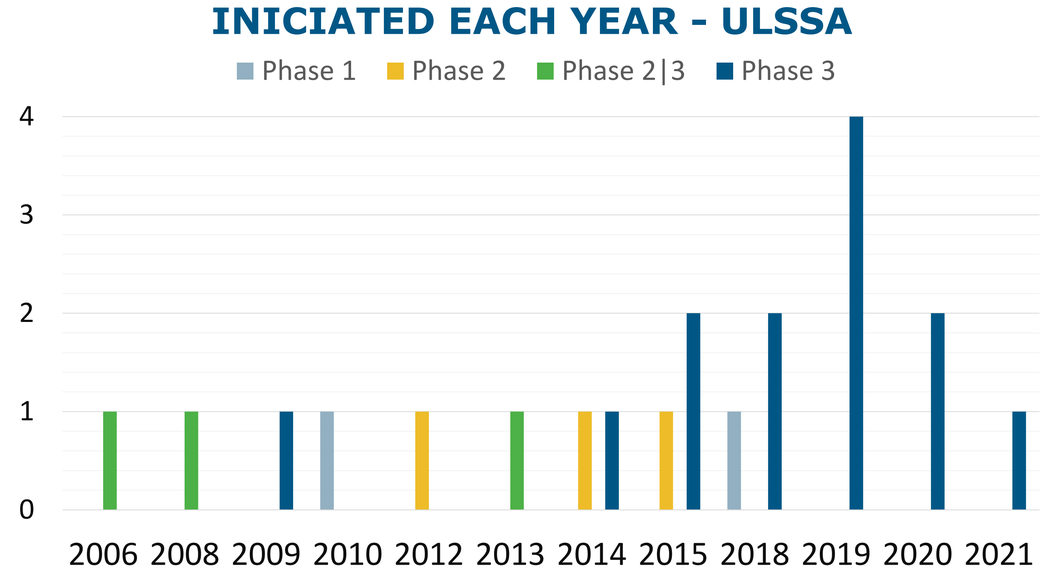


Among the approved therapeutic options are:

- **Transthyretin Stabilizers:**
  - Tafamidis: PDCTU participated in 3 phase 2/3 and 3 clinical trials.
- **Transthyretin Lowering Agents (Genetic Silencing):**
  - Patisiran: PDCTU participated in 7 phase 1, 2, and 3 clinical trials.
  - Inotersen: PDCTU participated in 2 phase 2/3 and 3 clinical trials.

The distribution of ULSSA's participation in new clinical trials related to hATTR in recent years unfolded as follows:

NUMBER OF CLINICAL TRIAL INICIATED EACH YEAR - ULSSA



CONCLUSION AND RELEVANCE

Investigational therapies for the treatment of hATTR are currently being evaluated, including agents that stabilize TTR, an investigational antibody, a subcutaneous antisense oligonucleotide, and an RNAi therapeutic.

Active clinical trials related to hATTR represent approximately 6% of the total active trials at ULSSA.

Each trial associated with hATTR had an average participation of 8 patients, a significantly higher average compared to the overall average of 2 patients per trial at ULSSA. Considering the worldwide prevalence of this condition (around 10,000 patients globally), these percentages underscore the importance of ULSSA and particularly the PDCTU in the development of therapeutic innovation.

REFERENCES AND/OR ACKNOWLEDGEMENTS

[1] Paramiloidose em Portugal e no mundo. SNS – Portal do SNS. https://www.sns.gov.pt/noticias/2021/04/29/paramiloidose-em-portugal-e-no-mundo/

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