6ER-027 ECONOMIC BENEFIT AND CLINICAL ADVANTAGES WITH THE INCLUSION OF PATIENTS IN CLINICAL TRIALS RELATED TO PARAMYLOIDOSIS

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Background and Importance Access to innovative medicines requires extensive and careful pharmaco-economic evaluation.

The inclusion of patients in Clinical Trials (CT) allows early access to new experimental medicines and considerable economic saving for the healthcare system.

Aim and Objectives Evaluate the economic benefit of including patients with hereditary transthyretin amyloidosis (hATTR) in clinical trials between 2018 and 2023.

Material and Methods Retrospective analysis of paramyloidosis-related clinical trials taking place at the centre since 2018. The data collected were the number of paramyloidosis-related CT, the number of patients included the time of participation in the CT and the average price of conventional treatment.

Results At our Clinical Trials Unit there are currently 6 Paramyloidosis-related CT underway, involving a total of 65 patients.

In economic terms, patient participation on ongoing CT related to Paramyloidosis has led to a cumulative saving of 15,667,487.98€ , compared to the costs of conventional therapy (tafamidis[1],inotersen[2] and patisiran[3]).

The distribution of annual savings was:

- 2019: 644.396,70€
- 2020: 2.447.335,64€
- 2021: 4.465.670,09€
- 2022: 4.206.997,00€
- August of 2023: 3.903.088,55 €

Conclusion and Relevance Participation in CT allows early access to new experimental therapies and contributes to the development of new drugs and/or new therapeutic indications. In Paramyloidosis, new agents like TTR stabilisers, subcutaneous antisense oligonucleotides and iRNA therapies are potential new alternatives.[4]

By participating in CT, centres obtain an extra source of funding. The participation of patients in CT also allows for a reduction in costs, through the preservation of financial resources and medication.

The savings generated by the participation in CT help to provide better care and an efficiency healthcare system.

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Conflict of Interest No conflict of interest.

6ER-028 REAL-WORLD TREATMENT PATTERN AND EFFECTIVENESS OF PIRFENIDONE AND NINTEDANIB IN PATIENTS WITH IDIOPATHIC PULMONARY FIBROSIS: A MULTI-INSTITUTIONAL STUDY IN TAIWAN

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Background and Importance Pirfenidone and nintedanib have been proven survival benefits and been currently approved for idiopathic pulmonary fibrosis (IPF). However, real-world comparison of effectiveness between two antifibrotics remains limited in Asia.

Aim and Objectives Our study was aimed to assess: (1) factors associated with the choice of pirfenidone versus nintedanib; (2) dose modification during treatment; (3) overall survival (OS).

Material and Methods We conducted a retrospective cohort study by using the largest multi-institutional electronic medical records in Taiwan. We included IPF patients newly receiving pirfenidone or nintedanib during 2018–2020. We followed up included patients to death, loss of follow-up or December 2022. The clinical factors included age, sex, lung function, biochemical data, comorbidities and co-medications. Multiple logistic regression analysis was used to assess factors associated with drug choice. Dose modification was assessed every 3 months by using dose intensity in follow-up period based on as-treated analysis. In OS analysis, we applied probability of treatment weighting (IPTW) and Cox regression model to enhance the comparability of study subjects and estimate hazard ratio (HR) between two treatment groups, respectively.

Results A total of 86 patients receiving pirfenidone and 142 patients receiving nintedanib. Mean age and Forced vital capacity (FVC) were 70.7 11.3 years and 68.8 17.4%, respectively. The use of nintedanib was positively associated with the patients with chronic kidney disease (CKD) (odds ratio: 2.1, 95% CI: 1.06 – 4.18). Dose reduction rate was similar between two groups (59.3% vs. 65.4%, P = 0.34). After a median of 25.5 months follow-up, nintedanib users were associated with worsen OS than pirfenidone users (adjusted HR: 2.07, 95% CI: 1.24 – 3.45).

Conclusion and Relevance Our study showed CKD patients were likely prescribed nintedanib. Pirfenidone users had association of better all-cause mortality than nintedanib users. Further studies are suggested to confirm our findings.

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Conflict of Interest No conflict of interest.

6ER-029 A SYSTEMATIC REVIEW OF COMBINED POLY (ADP-RIBOSE) POLYMERASE INHIBITOR AND ANDROGEN RECEPTOR ANTAGONISTS IN METASTATIC CASTRATION-RESISTANT PROSTATE CANCER

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