Orphan drugs (ODs) are designed to treat rare diseases (RD), which are those affecting a small number of people (prevalence <1/2.000 inhabitant).

**Purpose**

To assess economic impact of drugs used to treat RD in a hospital outpatient pediatric pharmacy (HOPP) and a pediatric day hospital (PDH).

**Material and Methods**

OBSERVATIONAL, RETROSPECTIVE, TRANSVERSAL STUDY

- **Population**
  Pediatric patients (<18 years old) as well as adults with cystic fibrosis. Chemotherapy treatments administered in the Oncology Day Hospital were excluded.

- **Study period**:
  January 2016 - December 2016, conducted at a third-level Maternal and Children University Hospital.

**Collected variables**

- Pharmaceutical drug, active ingredient, number of packages, real unit cost, consumption data, medical service and treating unit were collected from Pharmacy dispensing software.

- The Orphanet® database was used to classify the drugs in ODs (recognized as orphan by the European Union or abroad) or drugs without orphan designation.

**Results**

**Drugs**

- 410 drugs to treat RDs
- 42 were dispensed in the HOPP
- 9 were administered in the PDH

**Patients**

- 2442 patients received at least one drug to treat RDs
- 2044 patients from HOPP
- 562 patients from PDH
- 164 patients received treatment in both
- 441 (18.1%) were treated with ODs
- 420 patients (20.5%) in HOPP
- 23 patients (4.1%) in PDH
- *2 patients received treatment in both

**Economic impact**

- Drugs used to treat RDs accounted
  - 7.7 million €
  - 3.8 million € in HOPP
  - 3.9 million € in PDH
  - 4.2 million € (54.5%) in ODs
  - 36.3% of total pharmaceutical expenditure in drugs at HOPP
  - 71 % of total pharmaceutical expenditure in drugs at PDH

**The 5 drugs with greatest economic impact were:**

- **HOPP budget**
  - Bosentan
  - Adalimumab
  - Ivacaftor
  - Ataluren
  - Sildenafil

- **PDH budget**
  - Eculizumab
  - Idursulfase
  - Elosulfase
  - Galsulfase
  - Velaglucerase

**Conclusion**

1. Pharmacological treatment with ODs has a great impact on direct medical costs, involving more than 50% of total pharmaceutical expenditure. Although OD use is more common in the outpatient pharmacy than in the day hospital (20.5% and 4.1% of the assisted patients, respectively), the OD cost reaches 71% of the expenditure on drugs in the PDH setting.

2. HOPP and PDH need to develop strategies focusing on ODs, but also on treatments in special situations and extemporaneous drug formulations used to treat patients affected by RD.