4CPS-253

J01 - Antibacterials for systemic use

THE IMPORTANCE OF THE PHARMACOKINETIC PROFILE IN PATIENTS WITH ULTRA-RARE DISEASES:A CASE REPORT

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Background and importance

Mucopolysaccharidosis VII (MPSVII), also known as Sly syndrome, is an ultra-rare disease characterized by the deficiency of β-glucuronidase. Sly phenotypes vary from severe forms with hydrops fetalis and skeletal dysplasia, hepatosplenomegaly, heart valve abnormalities and mental retardation; to milder forms with fewer manifestations.

Aim and objectives

To compare the vancomycin pharmacokinetic profile observed in a newborn with MPSVII with the one expected in an average neonate.

Material and methods

- Clinical data: from electronical medical record (Diraya®)
- Literature research: from electronic databases (Pubmed, Scopus).

The serum concentration-time profiles were adjusted using the Abottbase PKSystem (PKS) program to a one-compartment neonatal population PK model incorporating body weight and renal function as the significant covariates.

Results

The patient was a 26 days-old male, with a postmenstrual age of 38 weeks, and diagnosed with MPS VII, who started with phlebitis and fever during his stay in the Neonatal Intensive Care Unit. His blood cultures were positive for coagulase-negative *Staphylococcus aureus*. The patient was treated with vancomycin 10 mg/kg/8h intravenously. The pharmacokinetics (PK) were evaluated before the sixth dose, with a weight of 2.2 kg and a height of 44 cm, and a creatinine serum level of 0.92 mg/L.

Initial treatment:
Vancomycin 10 mg/kg/8h intravenously.
Target: **trough range 10-15 mcg/mL.**

↓

Day 3:
Vancomycin serum level: **123.6 mcg/mL**

↓ Vancomycin stopped

Day 5:
Vancomycin serum level: **11.4 mcg/mL**

↓ Vancomycin restarted (10 mg/kg/12 h)

Day 7:
Vancomycin serum level: **48.2 mcg/mL**

Antimicrobial switched to cloxacillin →

Day 10:
The patient passed away due to complications related with his disease.

Pharmacokynetics parameters	Average	In this patient
Half-life	4-8 hours	15.8 hours
Distribution volume	1.8 L	1.99 L
Clearance	0.148 L/h	0.088 L/h

Conclusion and relevance

A 2-3 times greater half-life is observed in this patient with Sly syndrome. The large accumulation of vancomycin is not described in the literature neither expected with the features of this disease, highlighting the importance of therapeutic drug monitoring in patients with ultra-rare diseases, whose pharmacokinetics could be disturbed by factors still unknown.

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