SIROLIMUS FOR THE TREATMENT OF COMPLICATED VASCULAR ANOMALIES IN CHILDREN

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Background:

Vascular anomalies comprise a heterogeneous group of disorders. The presence of D2-40 markers and the Kasabach-Merrit phenomenom (KMP), are associated with a major gravity.

Objectives:

To analyze the efficacy and safety of treatment with sirolimus in children with complicated vascular anomalies (CVA).

Material and methods:

Retrospective observational study	December 2014-August 2016
Inclusion criteria: pediatric patients with CVA treated with sirolimus (off-label use)	Data collected: epidemiological and clinical caracteristics, treatment and evolution

Results:

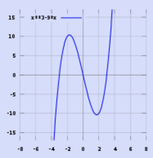
CASE 1

14-month-old male

Lymphangiomatosis in his right upper extremity

↓ Rehabilitation treatment failure

Sirolimus 0,8 mg/m^2/12h



Plasma level peak: 13,22 ng/mL [6,27- 26,19]

Dose adjustement: 0,4 mg/m2/day (azithromycin concomitance)

After 262 days with active treatment, objective clinical improvement in the functionality of the affected limb was achieved.

No adverse effects were observed

CASE 2

32-month-old male

Unresecable cervical kaposiform hemangioendothelioma KMP treated with acetylsalicylic acid + ticlopidine, previously treated with vincristine and systemic high-dose glucocorticoids

↓

Sirolimus 0,8 mg/m^2/12h → Plasma level peak: 9,86 ng/mL [3,49- 17,8]

Adverse effect: Hypertriglyceridemia

Dose reduction: 0,8 mg/m^2/day → Plasma level peak: 3,73 ng/mL [2,9- 4,95]

Platelet values at fifth day and maintained normal during all the treatment (388 days), and 88 days after stopping it.

Conclusions:

Sirolimus has been shown as an effective therapeutic option for CVA in childhood. It was well tolerated, and adjusting plasma levels allowed adverse effects minimisation without compromising effectiveness. Further studies are needed to determine the contribution of mTOR inhibitors in the treatment of childhood vascular anomalies.

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