CP-173

TOPICAL 0.1% RAPAMYCIN FOR ANGIOFIBROMAS IN A PAEDIATRIC PATIENT WITH TUBEROUS SCLEROSIS

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BACKGROUND

Facial angiofibromas (FA) are the most visible of the cutaneous manifestations of tuberous sclerosis. Current treatments include laser and other invasive techniques. Topical rapamycin is a recient and unauthorised option to treat FA (off label use) but a commercially available compound has not yet been developed.

OBJECTIVE

To evaluate the efficacy and safety of a pharmaceutical compounding of topical rapamycin in a child with FA.

METHODS

A retrospective review of the literature was conducted to select the vehicle, concentration and posology of the topical formulation. Topical **0.1%** rapamycin **in petrolatum** using the powder from the manufacturer was the pharmaceutical compounding selected. This concentration was proposed because is an effective, efficient and safety therapy in children pretreated. The vehicle selected to prepare this topical preparation was petrolatum because the treatment with topical rapamycin solution reported local adverse side effects as irritation. The treatment was authorised by the hospital management and child´s parents were informed and provide informed consent. The autors evaluated the efficacy through the improvement of the lesions and the safety was evaluated through the adverse effects at three months.

RESULTS

A 6 years old patient with FA was selected to treat with **topical 0.1% rapamycin in petrolatum** using the powder from the manufacturer **twice daily** to affected areas on the face.

In this patient there was a improvement and clearance of the lesions.

No irritation local and no serious adverse events were described.

A rapamycin blood level was taken at 3 months and had **1.02** ng/mL far below the therapeutic range (5-15 ng/mL) needed for immunosuppression.

The posology was **reduced** to **three times a week** instead of daily for maintenance.

CONCLUSION

✓ Topical 0,1% rapamycin in petrolatum has been an **effective treatment for FA in this patient**.
✓ The preparation formulated in petrolatum was **well tolerated with no adverse effects**.
✓ This pharmaceutical compounding could be used as an effective option for treatment of FA in paediatric patients without serious adverse effects.
✓ It is necessary to establish how long the treatment must be continued.