Title: Proposal for a new therapeutic high dosage of Pidotimod in children with Periodic fever, aphthous stomatitis, pharyngitis, adenitis (PFAPA) syndrome: a randomized controlled study.

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Abstract

Background. Despite to PFAPA syndrome is considered a benign and self-limited condition in childhood its impact on patients and families can be remarkable in many cases. Currently, the therapeutic options for managing are non-specific and no consensus exists about the best treatment to use. Pidotimod has been suggested as a new potential treatment in PFAPA syndrome for its immunodulatory effects. We conducted a preliminary, prospective, controlled, open, cross-over trial to assess the efficacy and the safety of Pidotimod in the treatment of children with PFAPA syndrome.

Methods. 22 children with PFAPA syndrome were randomly allocated to treatment with pidotimod (with 2 vials of 400mg daily) in combination with betamethasone 0.5-1 mg on need, based on parents/caregivers' decision (group A) or betamethasone 0.5-1mg on need, based on parents/caregivers' decision (group B). Each treatment period was for 3 months (Phase 1), after that patients were switched to the other arm for other 3 months (Phase 2). Efficacy was expressed in terms of number of episodes of fever, pharyngitis, or aphthous stomatitis, as well as the additional use of betamethasone on need. Safety and tolerability of the Pidotimod were evaluated on the basis of the number and type of adverse events (AEs) recorded during the treatment.

Results. Patients receiving Pidotimod and use betametasone showed a significant decrease in frequency of fevers (p=0.002); number of episodes of pharyngitis (p=0.049); aphthous stomatitis (p=0.036) as well as the betamethasone use on need (p=0.007). Overall, 19/22 (86.4%) showed benefits from Pidotimod administration. The safety profile of Pidotimod was excellent as no serious adverse events have been reported in the treated groups.

Conclusions. We firstly showed that high dosage of Pidotimod could be an effective and safe to reduce the PFAPA attacks in children.

Key words: betamethasone; children; high dosage; immunomodulation; PFAPA; pidotimod; treatment.