INTRODUCTION

Off-label and unlicensed use is widespread in the medical therapy of children. Since 2007 there is an EU regulation, which includes the need for a pediatric investigation plan (PIP) in the drug approval process. In 2018, hydrocortisone granules in capsules for opening (Alkindi®) became the first medicine to be approved for the treatment of adrenal insufficiency in children from birth until the age of 18.

AIM

To collect information on the underlying diagnoses, frequency of and compliance with glucocorticoid therapy, use of glucocorticoids during illness (stress dosing), and acceptance of or satisfaction with the hydrocortisone granules in capsules for opening in children with adrenal insufficiency.

METHOD

- Anonymized online survey (SurveyMonkey)
- Participants were recruited by informing them about the survey during their visits in the outpatient clinic and with the help of support-groups.

RESULTS

The following are preliminary results of the ongoing survey of parents of children aged 0-18 years.

- In 98% of those who have participated so far, children are treated by a pediatric endocrinologist.
- All children were treated with hydrocortisone as glucocorticoid therapy.
- The children of most participants were suffering from CAH (79%).
- 73% of the participants were satisfied with the new hydrocortisone preparation.

CONCLUSIONS

The hydrocortisone granules in capsules for opening show a good acceptance among parents, for whom simplicity of handling and accuracy of dosing are particularly important. The hydrocortisone granules are most often used for the treatment of younger children under 12 years of age in everyday use as well as for stress dosing.

REFERENCES

Neumann U. et al. A Prospective Study of Children Aged 0-8 Years with CAH and Adrenal Insufficiency Treated with Hydrocortisone Granules. J Clin Endocrinol Metab. 2021;106(3):e1433-e1440