Study to assess the knowledge of caretakers regarding corticosteroid therapy in children with congenital adrenal hyperplasia – 21 hydroxylase deficiency

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ABSTRACT

Background: The primary aim of treating congenital adrenal hyperplasia (CAH) due to 21 hydroxylase deficiency is to replace the deficient glucocorticoids and mineralocorticoids, to minimize the excess androgen production and to facilitate normal growth. Children with CAH require daily treatment lifelong and increased dosage plan during acute stress, in order to obtain the benefit of optimal outcome from the ongoing treatment schedule. This emphasizes the need for the parents of affected children to be empowered with adequate knowledge regarding such lifesaving therapy. Aims & Objectives: This study was aimed to assess their knowledge regarding corticosteroid therapy. Materials and methods. Caretakers of children with CAH were recruited by using purposive sampling technique. Data was collected by using structured interview technique. Results: It was observed that only 10% of study group had adequate knowledge about therapy and the majority of them were from rural areas, lacking in awareness of essential steps of management. Conclusion: It was concluded that these caretakers need to be given appropriately planned education regarding corticosteroids, drug actions, and the need for enhanced steroid dosage during stress situations. They should also be given relevant instructional materials to read for improving their knowledge about their child’s disorder.

Keywords: 21 hydroxylase deficiency, congenital adrenal hyperplasia, parenting, steroid therapy

Introduction

Children with congenital adrenal hyperplasia (CAH) due to 21 hydroxylase deficiency cannot be cured at present, but as long as they receive adequate care and regular treatment, they can lead normal, fulfilling lives. It is an inherited genetic defect in the adrenal cortex which fails to produce corticosteroids and this results in reduced production of glucocorticoids (cortisol) and mineralocorticoids (aldosterone) with a simultaneous excess of adrenal androgens.[1] Cortisol serves a vital physiologic function of maintaining homeostasis and more importantly, helps to combat infections and stressful situations. It is also responsible for maintaining blood sugar levels, which, in turn, is essential to prevent mental confusion and seizures arising from hypoglycaemia. Besides, cortisol is responsible for maintaining normal blood pressure.[2]

For more than six decades now, glucocorticoid therapy has been undoubtedly beneficial as a life-saving drug in the treatment of CAH and thousands of children have survived into adulthood. Treatment of classical CAH is commenced soon after birth and is essential to be continued throughout the patient’s life. Daily oral treatment with glucocorticoids (to replace cortisol), mineralocorticoids (to replace aldosterone), and salt supplements are prescribed, particularly in infancy. Throughout the child’s life,
periodic monitoring of serum hormonal profile is essential as this helps in arriving at the correct treatment dosage for optimal treatment, which, in turn, is crucial to prevent adrenal crisis and virilisation. However, the injudicious use of hydrocortisone may also lead to several undesirable long-term complications, including short stature, precocious puberty, obesity, hypertension, polycystic ovary syndrome, infertility, tumour, osteoporosis, and reduced quality of life. Since during times of high stress or illness, the adrenal glands are normally much more active, to supply more cortisol to help the body meet increased demands and recover rapidly from a life-threatening situation, CAH patients require close monitoring and increased hydrocortisone dosages during illness or after major surgery.

To achieve all the ideals of management, the parents of children with CAH need to be empowered with adequate knowledge regarding the merits of hydrocortisone therapy in this disorder and require support from the team of healthcare providers. Hence, this study was undertaken with the primary objective of assessing the parents’ knowledge regarding corticosteroid therapy and the risk of discontinuation of treatment which may result in a highly detrimental ‘adrenal crisis’ and to plan for an ongoing interventional programme for them. The association between parents’ knowledge and baseline variables were also analysed.

Material and Methods

The study was done in the Paediatric Endocrine Outpatient department in a selected hospital in Bengaluru. The research approach adopted for this study was quantitative and descriptive cross-sectional design. Thirty caretakers of children with CAH were recruited by using a purposive sampling technique. Data were collected by using a structured interview schedule. The study was started after obtaining permission from the IEC and IRB authorities. Informed consent was obtained from the caretakers after the study was explained to them. A questionnaire was used to collect data from the caretakers by interview method.

Population

The present study population comprised of all caretakers of children and adolescents between the ages of 1 and 18 years, with CAH 21 hydroxylase deficiency, who could understand English and Kannada and who were willing to participate in the study.

Sample

Samples were selected by using a purposive sampling technique. Thirty caretakers of children who sought treatment for CAH 21 hydroxylase deficiency from the Paediatric Endocrinology OPD of a selected hospital in Bengaluru were chosen for the study.

Criteria for selection

Inclusion criteria: Caretakers of children and adolescents between the ages of 1 and 18 years with CAH 21 hydroxylase deficiency, who could understand English and Kannada and were willing to participate in the study were included.

Exclusion criteria: Caretakers of children with CAH 21 hydroxylase deficiency who were diagnosed for the first time and not yet started on corticosteroid therapy.

Development of the tool

The tool was developed based on a review of the literature and experts’ opinions.

Description of the tool

The tool consisted of a structured questionnaire that was divided into two sections.

Section A: Baseline demographic variables containing name, age, and other social information. Section B: Knowledge questionnaire on corticosteroid therapy developed and validated by experts, consisted of eight multiple-choice questions with three options each and four open-ended questions. Each right answer was allotted one mark. The total score was 12.

Data analysis

Data analysis were done by using descriptive and inferential statistical method. The frequency and percentage were calculated to show the distribution of subjects according to baseline variables. The Student’s ‘t’ test was used to assess the association between parents’ knowledge scores and baseline variables.

Results

Out of the 30 caretakers included in the study, 36.7% were high school educated and 53.3% were mothers and 10% were guardians. The duration of illness in the children was more than 1 year. The majority of parents (63.34%) belonged to the age group of >35 years and 33.33% were between 26 and 35 years; 80% belonged to rural areas. Nearly a third (36.7%) had high school education and 33.4% were graduates [Table 1]. Children studied were equally distributed in the different age groups with no significant differences [Table 2].

Among the children, 46% were preschoolers and 70% were boys. Concerning knowledge of caretakers, it was observed that 10% had
adequate knowledge, 80% had moderate knowledge, and 10% had inadequate knowledge. The mean knowledge score is 7.63 [Table 3]. It was observed that 53% of the caretakers did not know the rationale behind why the drug should be taken after food. Nearly three-fourths (73%) of the caretakers did not know about follow-up.

**Discussion**

It is widely known that CAH does not have a cure currently but for many decades now, it is well-established worldwide that this disorder can be effectively treated to enable affected children to grow into adulthood and to lead normal fulfilling lives. The treatment in classic CAH begins from birth and is continued throughout life with regular monitoring of clinical and laboratory parameters to help in adequate optimal drug dosing, thereby avoiding side effects. An experienced paediatric team of specialists including endocrinologists, surgeons, clinical geneticists, radiologists, biochemists, psychiatrists, and others is essential for a successful outcome of management. Thus, the need of the parents of affected children to have sound knowledge of their child’s condition becomes paramount. While city-dwelling parents with sufficient means of income and education may avail such healthcare amenities with ease, the poorer parents with lesser income and education, hailing from rural areas will remain lacking in essential information regarding effective management and are unable to provide proper care essential to their affected children. Such parents who are often young, are influenced by myths and misconceptions conveyed to them by the elders in the immediate community and are also more vulnerable to suffer from unfounded fears. In general, they are led by lay public to doubt the very efficacy of any long-term drug treatment, however essential it may be.[4] The present study focused on this group of caretakers to assess their knowledge and attitude of CAH in their child and has yielded pilot data to undertake a study in the future to improve their awareness and coping skills.[5]

Nearly two-thirds of the parents are young, hailing from a rural background (80% - Table 3C) and not fully educated (~50% – Table 1). Knowledge about CAH and its treatment was assessed by using simple, easy-to-understand questions and was found adequate only in 10% of parents studied [Table 3A]. Out of 12 questions to assess the knowledge regarding corticosteroid therapy, 100% responded correctly for the route of administration, but only 16.67% knew correctly as to why the drug should not be stopped suddenly [Table 3B].[6]

For children with CAH, the provision of life-saving treatment which is highly effective, cost-effective, and not exorbitantly expensive should be within the purview of primary health care, as these children can lead normal lives, by receiving the suitable

### Table 2: Distribution of children according to the level of education, age, and duration of illness (n=30)

| Age         | Frequency | Percentage |
|-------------|-----------|------------|
| 1-3 years   | 7         | 23.33      |
| 4-6 years   | 7         | 23.33      |
| 7-12 years  | 12        | 40         |
| 13-19 years | 4         | 13.34      |

| Education     | Frequency | Percentage |
|---------------|-----------|------------|
| Toddlers/preschool | 8           | 26.6       |
| Primary school | 16        | 53.4       |
| Middle school  | 3         | 10         |
| High school    | 2         | 6.7        |
| Intermediate   | 1         | 3.3        |

| Duration of illness | Frequency | Percentage |
|---------------------|-----------|------------|
| 1-3 years           | 7         | 23.33      |
| 4-6 years           | 7         | 23.33      |
| 7-12 years          | 12        | 40         |
| 13-19 years         | 4         | 13.34      |

### Table 3: (A) Description of knowledge scores of caretakers

| Knowledge scores of caretakers | Frequency | Percentage |
|-------------------------------|-----------|------------|
| Good > 75%                    | 3         | 10%        |
| Average> 50-75%               | 24        | 80%        |
| Poor < 50%                    | 3         | 10%        |

### Table 3: (B) Distribution of caretakers by the correct response to each item in the questionnaire

| Item                                                                 | Correct response % | Wrong Response% |
|---------------------------------------------------------------------|--------------------|-----------------|
| The drug child is getting                                            | 93                 | 7               |
| Why drug must not be taken in empty stomach                          | 47                 | 53              |
| Best time of the day to take steroids                                | 76, 66             | 23, 33          |
| How can the drug treatment cause risk to the child                   | 44                 | 56              |
| What can you do when 1 dose is missed                                 | 95                 | 5               |
| Possible ways to minimise the toxic effect                            | 93                 | 7               |
| How must the drug be taken (route)                                    | 100                | 0               |
| How often must follow up be done for children with CAH               | 27                 | 73              |
| How does drug help your child                                        | 57                 | 43              |
| Dosage of drug                                                        | 30                 | 70              |
| When the dosage of the drug should be doubled                        | 43                 | 57              |
| Why drug should not be stopped suddenly                               | 16.67              | 83, 33          |

### Table 3: (C) Association between Knowledge scores of caretakers with baseline variables

| Baseline variables | Mean scores | Standard deviation | r | Test of significance |
|--------------------|-------------|--------------------|---|----------------------|
| Area of residence  | 7.41       | 1.414              | 0.41178 | dof=29 P=0.06 |
| Rural              | 7.41       | 1.414              | 0.41178 | dof=29 P=0.06 |
| Urban              | 8.79       | 1.78               | 0.6125 | dof=29 P=0.05 NS |
| Sex of caretakers  | 7.47       | 1.61               | 0.6125 | dof=29 P=0.05 NS |
| Male               | 7.47       | 1.61               | 0.6125 | dof=29 P=0.05 NS |
| Female             | 7.44       | 1.68               | 0.7215 | dof=29 P=0.05 NS |
| Sex of child       | 7.57       | 1.69               | 0.7215 | dof=29 P=0.05 NS |
| Male               | 7.57       | 1.69               | 0.7215 | dof=29 P=0.05 NS |
| Female             | 7.44       | 1.68               | 0.7215 | dof=29 P=0.05 NS |

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treatment. With this experience, a larger study is planned to devise a comprehensive interventional study for parents of children with CAH. All aspects of the disorder, including awareness of the condition, the basis of regular compliance with no interruptions in treatment, the importance of stress dosing, and the advantages of regular follow-up will need to be emphasised in a future study.

**Conclusion**

The study shows that only 10% of parents of children with CAH had adequate knowledge for managing their children effectively. Hence, there is an urgent need to provide essential information to these parents, in the form of group sessions, relevant printed instructional material for reading, and organisation of refresher meetings from time to time for achieving the desired goal of normal growth and good quality of life among children and adolescents with CAH.

**Key message**

Parents of children with CAH require to be aware of many details regarding the disorder, the treatment involved for lifelong therapy, stress dosing to overcome life-threatening situations, the regularity of monitoring and followup. Greater effort is urgently needed to improve their knowledge regarding CAH to ensure optimal management and outcome.

**Declaration of patient consent**

The authors certify that they have obtained all appropriate patient consent forms signed by caretakers of children with CAH. In the form, the caretaker(s) has/have given his/her/their consent for clinical information to be reported in the journal. The caretakers understand that their names and initials will not be published and due efforts will be made to conceal their identity, but anonymity cannot be guaranteed.

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**Conflicts of interest**

There are no conflicts of interest.

**References**

1. Charmandari E, Nicolaides NC, Chrousos GP. Adrenal insufficiency. Lancet. 2014; 83:2152-67.
2. Hsu CY, Rivkees SA. Congenital adrenal hyperplasia: A parents’ guide. Author House. Bloomington, Indiana. 2005.
3. Simpson A, Ross R, Porter J, Dixon S, Whitaker MJ, Hunter A. Adrenal Insufficiency in Young Children: a Mixed Methods Study of Parents’ Experiences. J Genet Couns 2018;27, 1447–1458.
4. Bhakhri BK, Jain V. Congenital adrenal hyperplasia: as viewed by parents of affected children in India - a pilot study. J Pediatr Endocr Met 2011; 24: 959-963.
5. de Silva K.S.H, de Zoysa P, Dilanka W.M.S, Dissanayake B.S. Psychological impact on parents of children with congenital adrenal hyperplasia: a study from Sri Lanka. J Pediatr Endocr Met 2014;27:475‑8.
6. Joshi P, Yadav B, Jain V, Sharma S. Knowledge, stress and adopted coping strategies of parents of children having congenital adrenal hyperplasia: An exploratory survey. Indian Journal of Child Health. 2017; 4:127-32.
7. Vidmar AP, Weber JF, Monzavi R, Koppin CM, Kim MS. Improved medical-alert ID ownership and utilization in youth with congenital adrenal hyperplasia following a parent educational intervention. J Pediatr Endocrinol Metab 2018;31:213-219.