

Development of a novel weight-based steroid emergency plan for patients with Duchenne Muscular Dystrophy

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INTRODUCTION

- Duchenne Muscular Dystrophy (DMD) is a rare, X-linked recessive disorder due to mutations in the dystrophin gene.^{1,2} It affects 1 in 3000-7000 live male births worldwide and is characterised by inflammation and progressive degeneration of skeletal and cardiac muscle.^{1,3}
- Long-term glucocorticoid therapy aims to reduce disease progression and standard of care may utilize Prednisolone or Deflazacort (an ozazoline derivative of prednisolone), which have both been shown to improve outcomes in patients with DMD.^{3,4,5}
- Recommended glucocorticoid doses however are supraphysiological, suppressing the hypothalamo-pituitary-adrenal axis, and resulting in adrenal insufficiency.
- All boys prescribed glucocorticoid therapy should be assumed to have adrenal suppression, and are therefore at risk of adrenal crisis during illness or stress (e.g. surgery, bisphosphonate infusions).
- The updated DMD Care Considerations consensus document (2018) recommends implementation of emergency plans⁶ however unified national or international guidance for illness management for this cohort does not exist..
- Previously emergency sick day plans were not consistently in place within our own specialist centre, and on national survey of 6 other NHS Trusts, it was clear that advice and education for “sick day” steroid regimens was variable.

RESULTS

- A novel weight-based regimen of “sick day” oral hydrocortisone dosing (aiming for ~15-20mg/m²/day given as 4 equal divided doses) was developed using the ‘Body Surface Area in Children’ charts from the British National Formulary for Children.⁷

| WEIGHT KG | ORAL HYDROCORTISONE DOSE TO BE GIVEN QDS AT 06:00/12:00/18:00/24:00 | BSA | Lower total daily dose mg/m ² /day | Upper total daily dose mg/m ² /day |
|-----------------------------------|---|-------------|---|---|
| UNDER 10KG OR UNDER 1 YEAR OF AGE | DISCUSS WITH ENDOCRINE TEAM | | | |
| 10 - 15 | 2.5 MG | 0.49 – 0.65 | 15 | 20 |
| 16 - 34 | 5 MG | 0.68 – 1.1 | 18 | 29 |
| 35 - 60 | 7.5 MG | 1.2 – 1.7 | 17 | 25 |
| 61+ | 10 MG | 1.7 – 2.2 | 18 | 23 |

- The 6 hourly dosing of hydrocortisone is given **in addition** to the patients long term glucocorticoid regimen to ensure 24 hour steroid cover during illness / stress, and to prevent any impact on underlying disease control.
- Training and education materials were produced to increase awareness of adrenal insufficiency among patients, families and staff.
- All families and boys are educated about “sick day” dosing plans, as well as how to recognise signs/symptoms of an adrenal crisis. They have been trained to give an IM Hydrocortisone injection. The families have written plans for sick day and crisis management for home, as well as plans for school and the local hospital.
- In 2020, roll out of face to face training was rapidly converted to remote training and videos were produced due to COVID19 restrictions. Face to face injection training refresher sessions are now provided when families are seen in clinic.
- Adrenal ‘Flags’ have been added to our electronic patient records alerting staff that these patients are at risk of adrenal crisis.



AIM

- We sought to develop a weight-based steroid emergency regimen to cover sickness/stress for DMD patients managed within our tertiary specialist centre.
- Many of this cohort are non-ambulant, and accurate measurements for body surface area can be challenging.

METHODS

- A novel weight-based regimen of “sick day” oral hydrocortisone given 6 hourly was devised.
- This was to be implemented when needed, in addition to the glucocorticoid the patient was taking for disease.

CONCLUSIONS

- We are currently reviewing the results of our practice change. A Trust wide programme for recognizing and treating adrenal crisis has been developed and implemented for ward based nurses and this cohort has become the pilot for the development of new Trust protocol for adrenal suppression.
- Anecdotally families report faster recovery times from illness since using sick day doses of hydrocortisone alongside their usual glucocorticoid for DMD.
- Previous emergency sickness/stress regimens in this cohort, often utilising a patient’s usual disease-modifying glucocorticoid, may not have taken into account the pharmacokinetics of these drugs
- The development of a unified, consistent approach to illness management, alongside structured education for DMD patients and families, are initial actions towards improving clinical outcomes. Further guidance at a national and international level is needed.

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