

CORTICOSTEROIDS IN DUCHENNE MUSCULAR DYSTROPHY: DMD Care UK standard of care guideline, March 31st 2025

Appendix B: FOR DMD: Precautionary Interventions from Guglieri et al, 2022 (see footer)

<https://pmc.ncbi.nlm.nih.gov/articles/PMC8984930/>

BEHAVIOR CHANGES

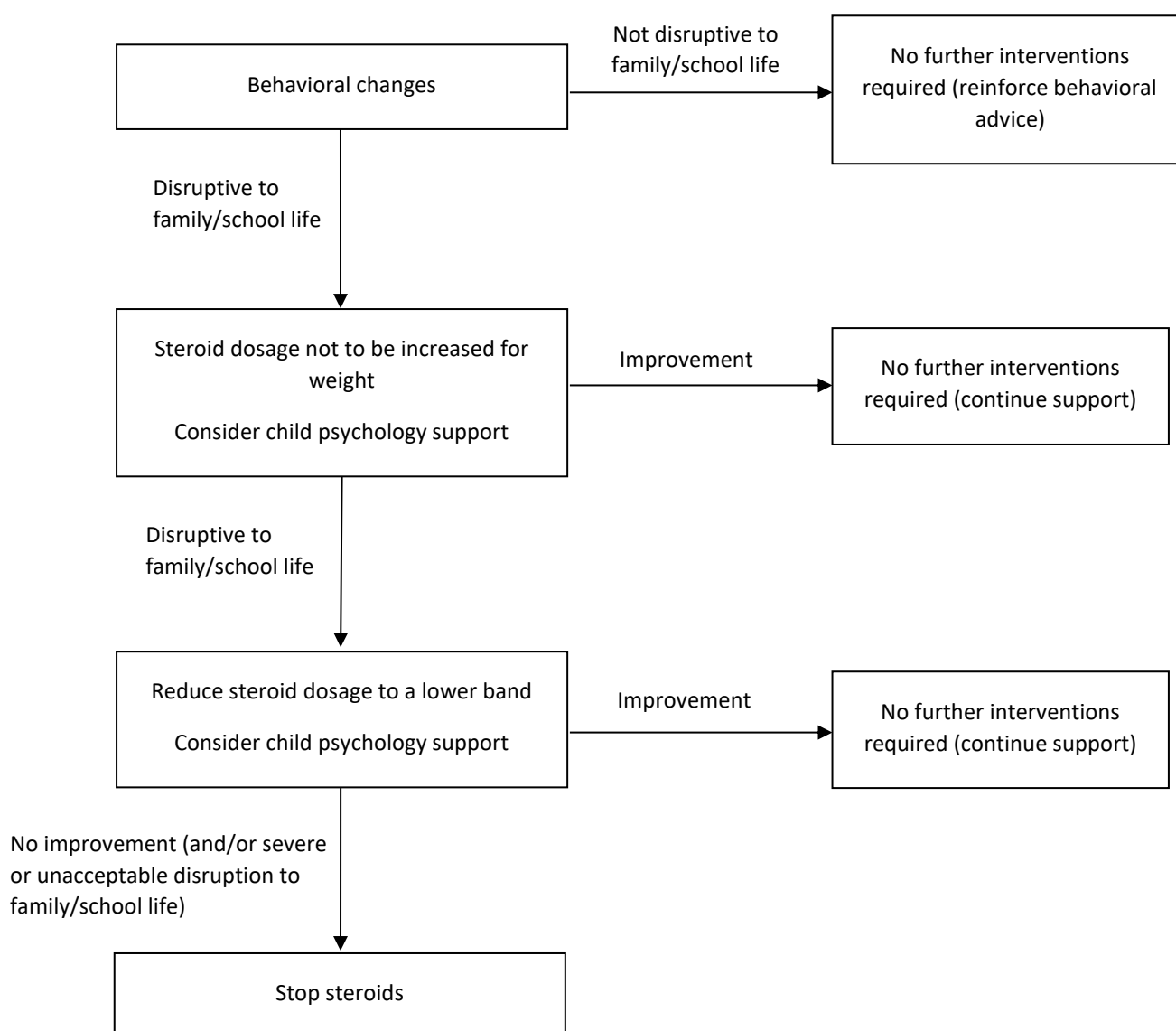
Prophylactic measure:

Standardized behavioral advice at baseline/entry visit and at all follow-up visits.

Assessment:

Parent(s)/guardian(s) completion of SDQ, PARS III, IOWA Conners, Revised Rutter scale at baseline and at each follow up visit.

Discussion with parents/guardians on how they perceive differences in their child's behavior at each follow up visit.

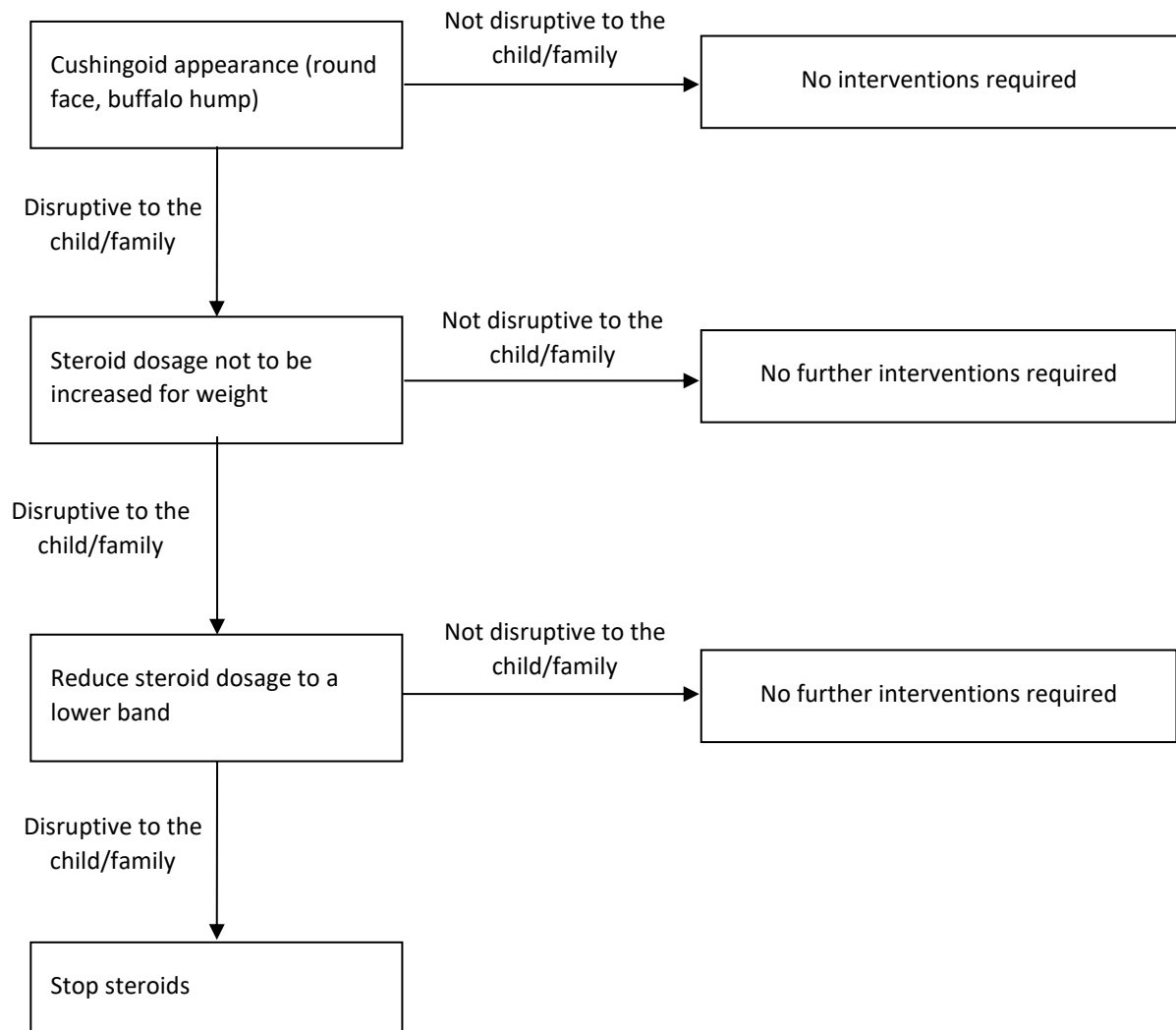


Adapted (with permission) from: Guglieri M, Bushby K, McDermott MP, Hart KA, Tawil R, Martens WB, Herr BE, McColl E, Speed C, Wilkinson J, Kirschner J, King WM, Eagle M, Brown MW, Willis T, Griggs RC; FOR-DMD Investigators of the Muscle Study Group; Straub V, van Ruiten H, Childs AM, Ciafaloni E, Shieh PB, Spinty S, Maggi L, Baranello G, Butterfield RJ, Horrocks IA, Roper H, Alhaswani Z, Flanigan KM, Kuntz NL, Manzur A, Darras BT, Kang PB, Morrison L, Krzesniak-Swinarska M, Mah JK, Mongini TE, Ricci F, von der Hagen M, Finkel RS, O'Reardon K, Wicklund M, Kumar A, McDonald CM, Han JJ, Joyce N, Henricson EK, Schara-Schmidt U, Gangfuss A, Wilichowski E, Barohn RJ, Statland JM, Campbell C, Vita G, Vita GL, Howard JF Jr, Hughes I, McMillan HJ, Pegoraro E, Bello L, Burnette WB, Thangarajh M, Chang T. Effect of Different Corticosteroid Dosing Regimens on Clinical Outcomes in Boys With Duchenne Muscular Dystrophy: A Randomized Clinical Trial. JAMA. 2022 Apr 19;327(15):1456-1468. doi: 10.1001/jama.2022.4315. PMID: 35381069; PMCID: PMC8984930.

CUSHINGOID APPEARANCE

Assessment:

Cushingoid appearance (round face and buffalo hump) will be assessed by the site investigator at the baseline/entry visit (T0) and at all follow up visits.



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GASTROINTESTINAL SYMPTOMS (ABDOMINAL PAIN, HEARTBURN, GI BLEEDING)

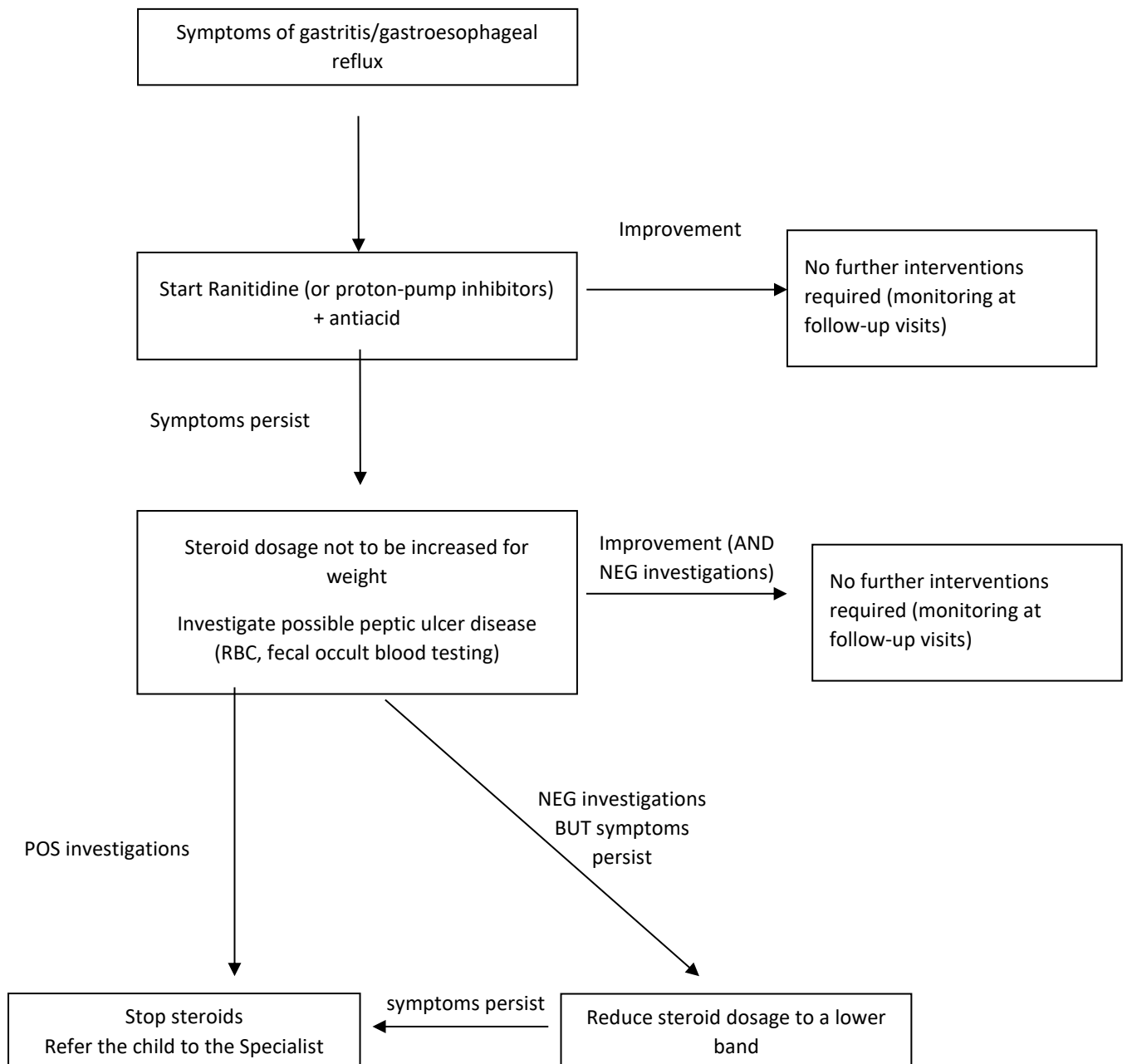
Precautionary interventions:

Avoid non-steroidal anti-inflammatory drugs.

Recommendation to take study medication on full stomach (after breakfast).

Assessment:

Physical examination at screening, baseline and at all follow up visits.



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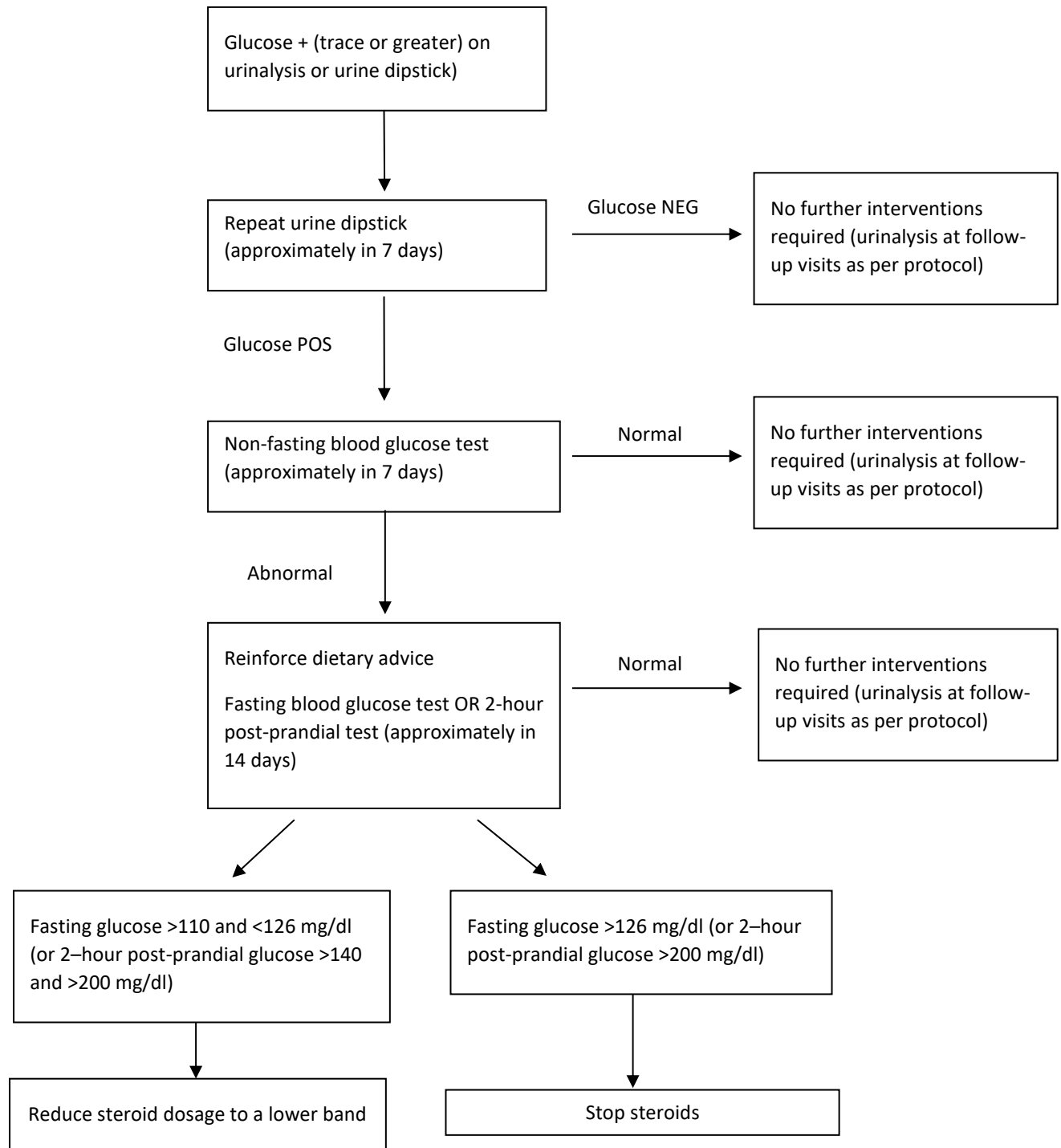
GLYCOSURIA

Precautionary interventions:

Standardized dietary advice.

Assessment:

Urinalysis for glycosuria at screening, baseline/entry visit and at all follow up visits.



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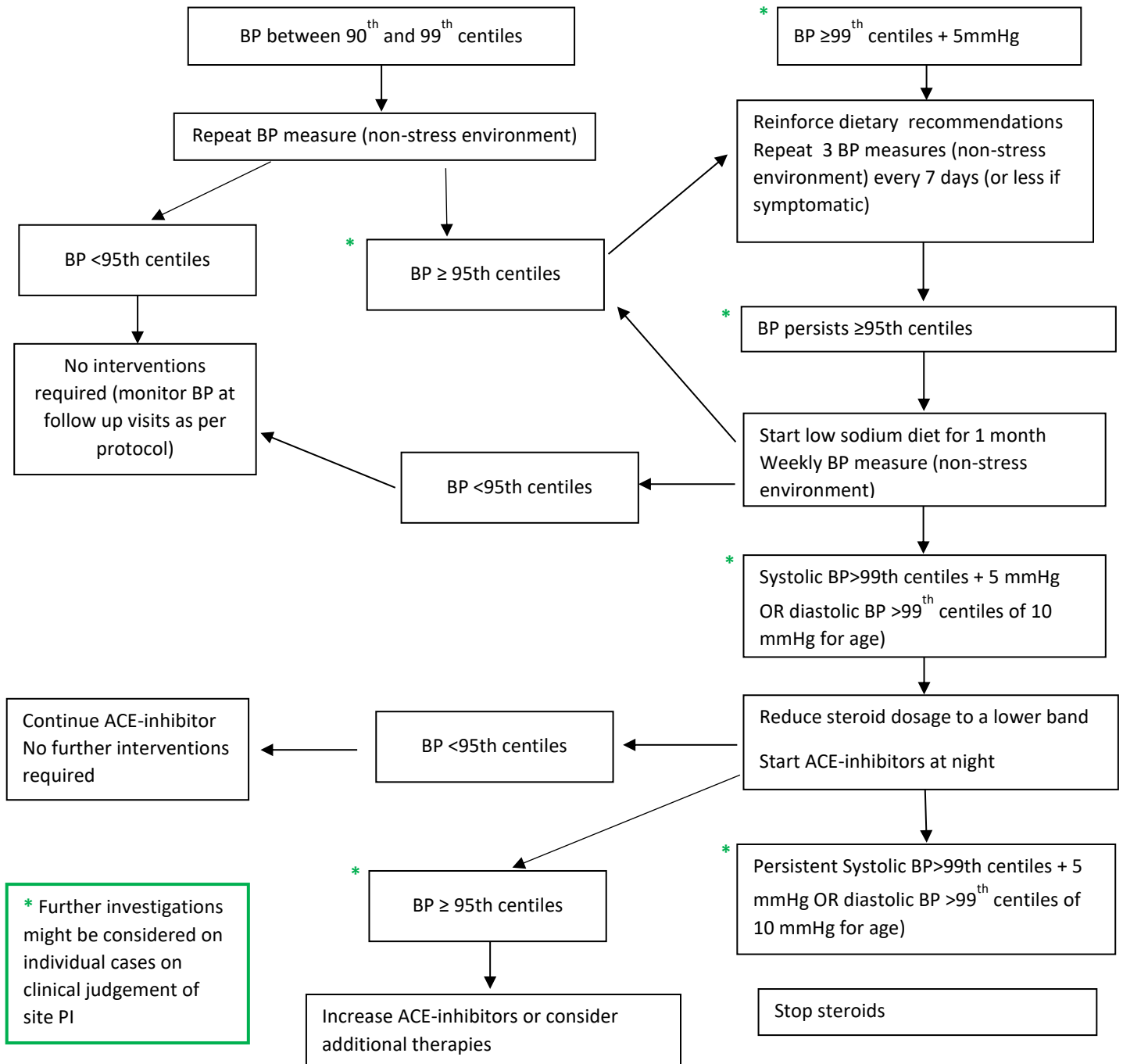
HYPERTENSION

Precautionary interventions:

Standardized advice about dietary sodium intake and weight control

Assessment:

BP measurements at screening, baseline/entry visit and at all follow up visits



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IMMUNO-ADRENAL SUPPRESSION

Precautionary interventions:

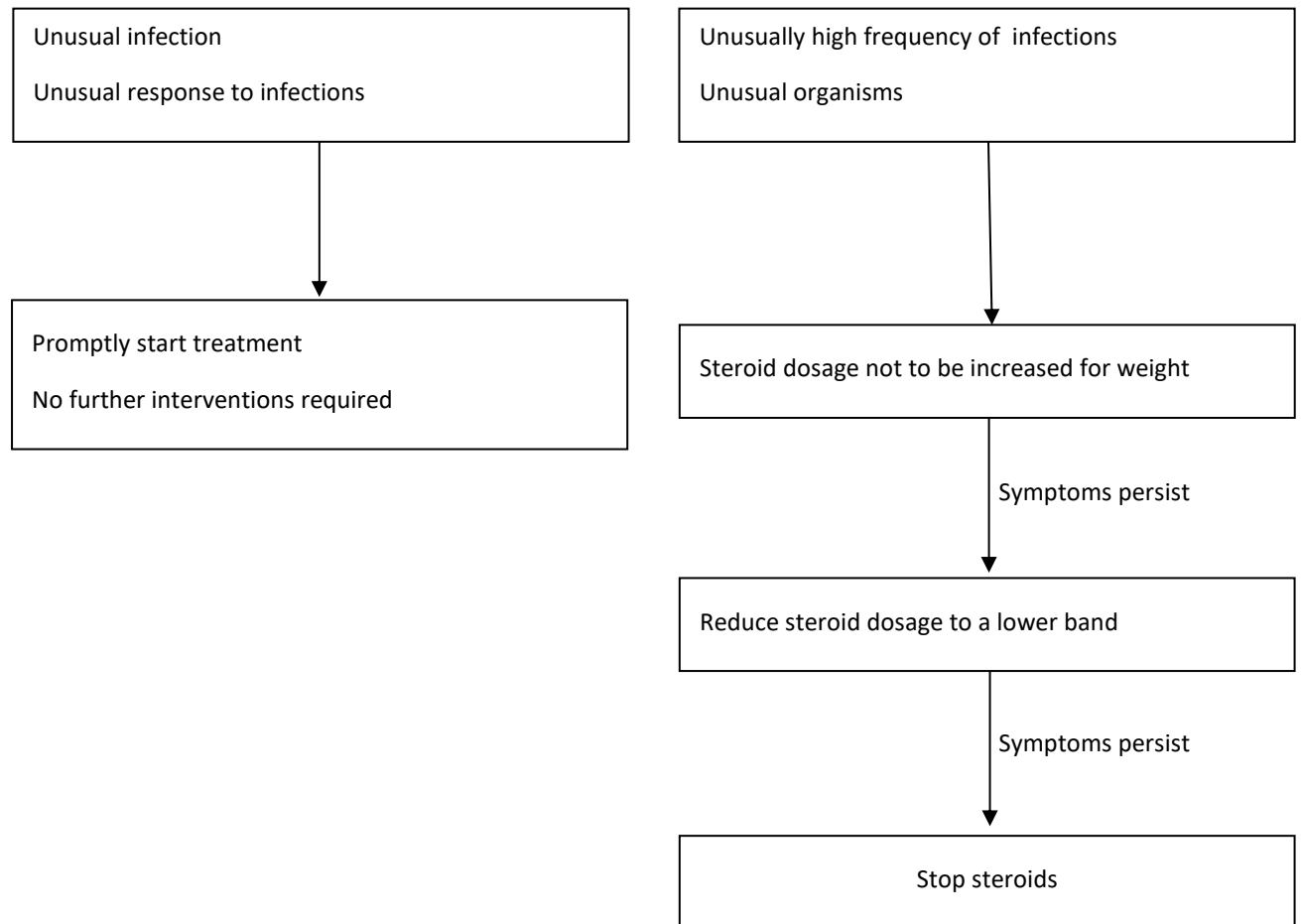
Chicken pox immunization (TBC immunization in at risk children)

Advice of promptly addressing infections

Advice on corticosteroid cover in case of illness, injury or surgery

Advice regarding progressive reduction of corticosteroids if discontinuation is required

Disallowing live vaccines during treatment

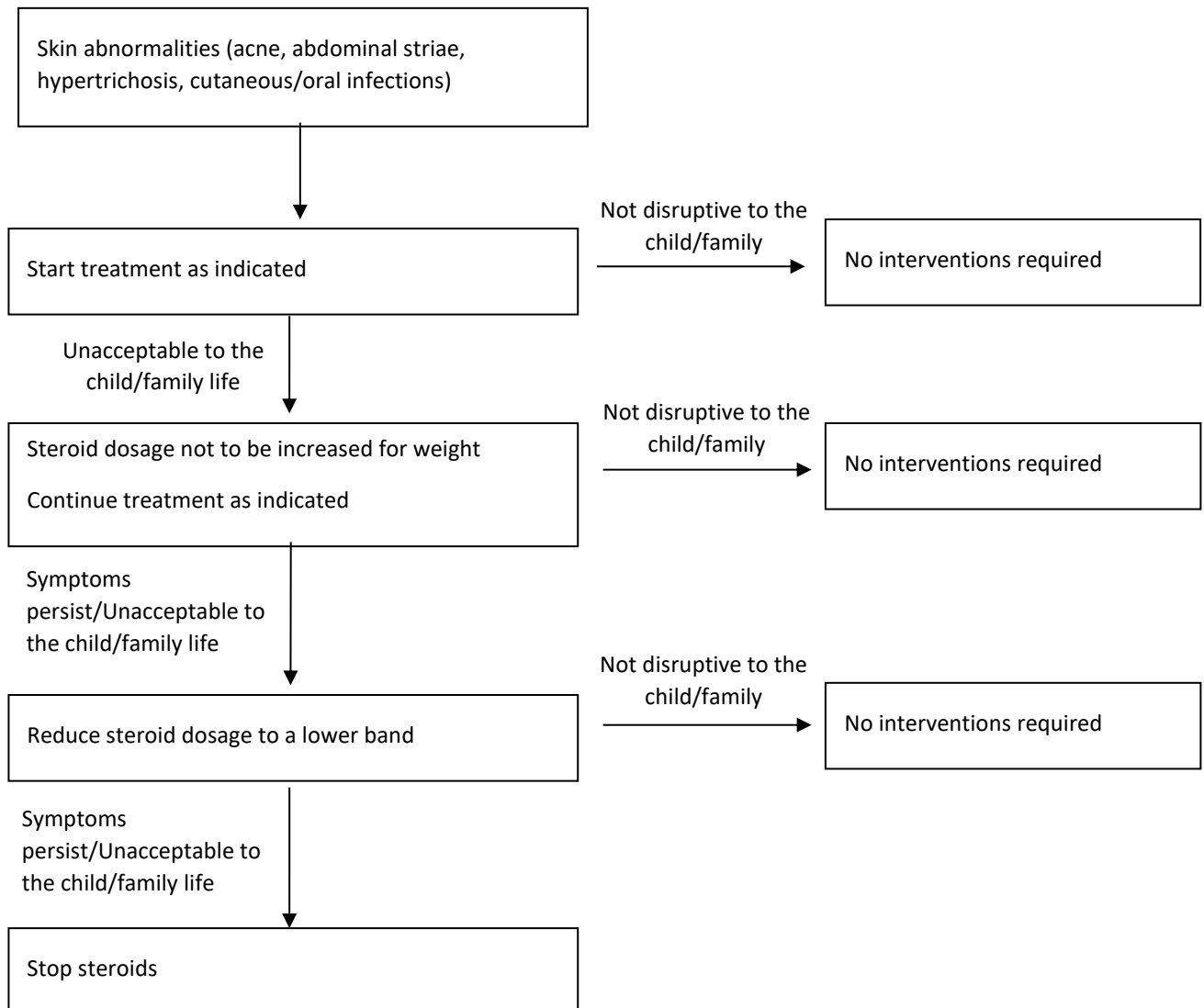


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SKIN CHANGES

Assessment:

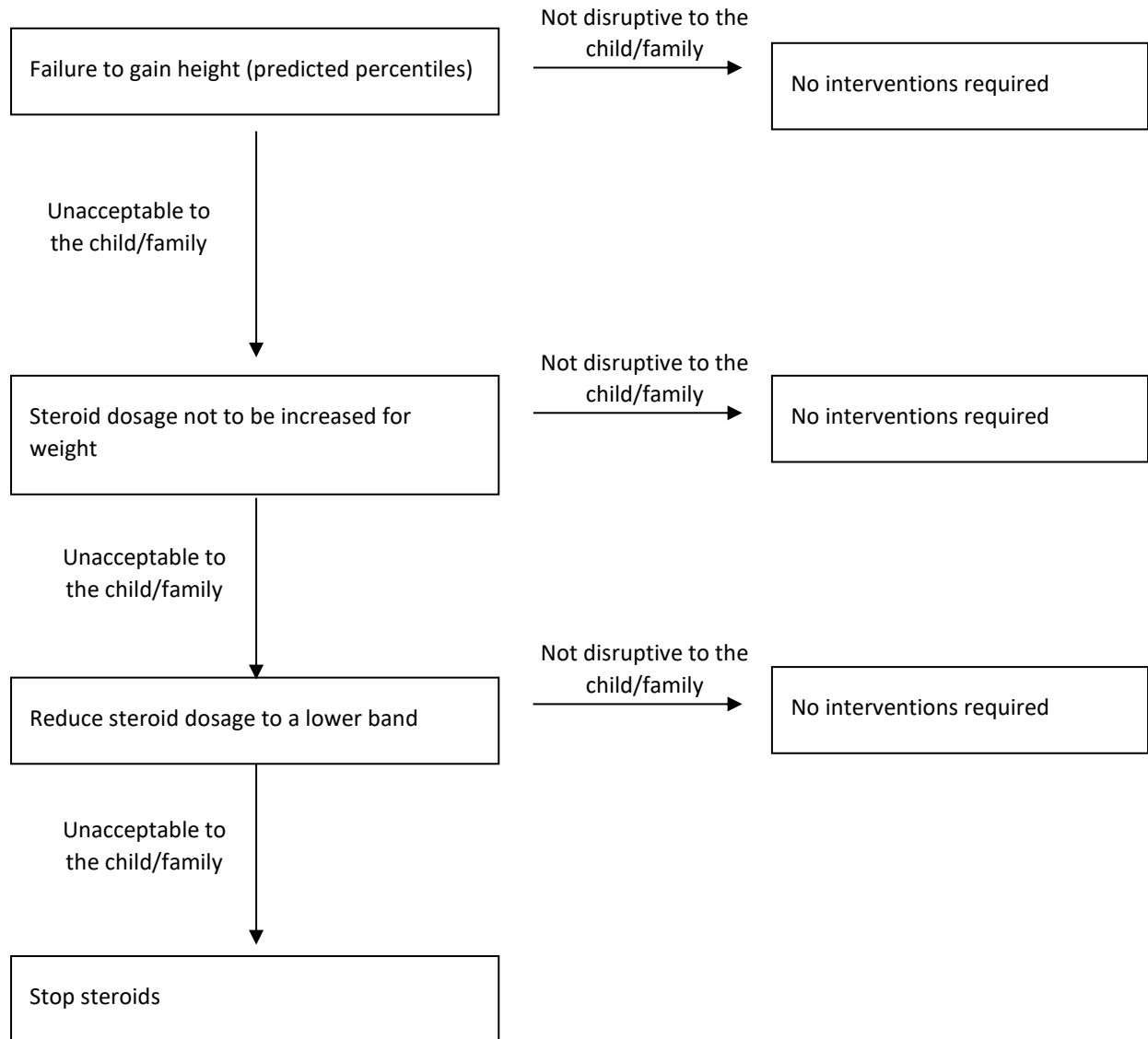
Skin examination at baseline/entry visit and at each 6-monthly follow up visit.



SLOW GROWTH

Assessment:

Height measured at screening, baseline/entry visit and at all follow up visits



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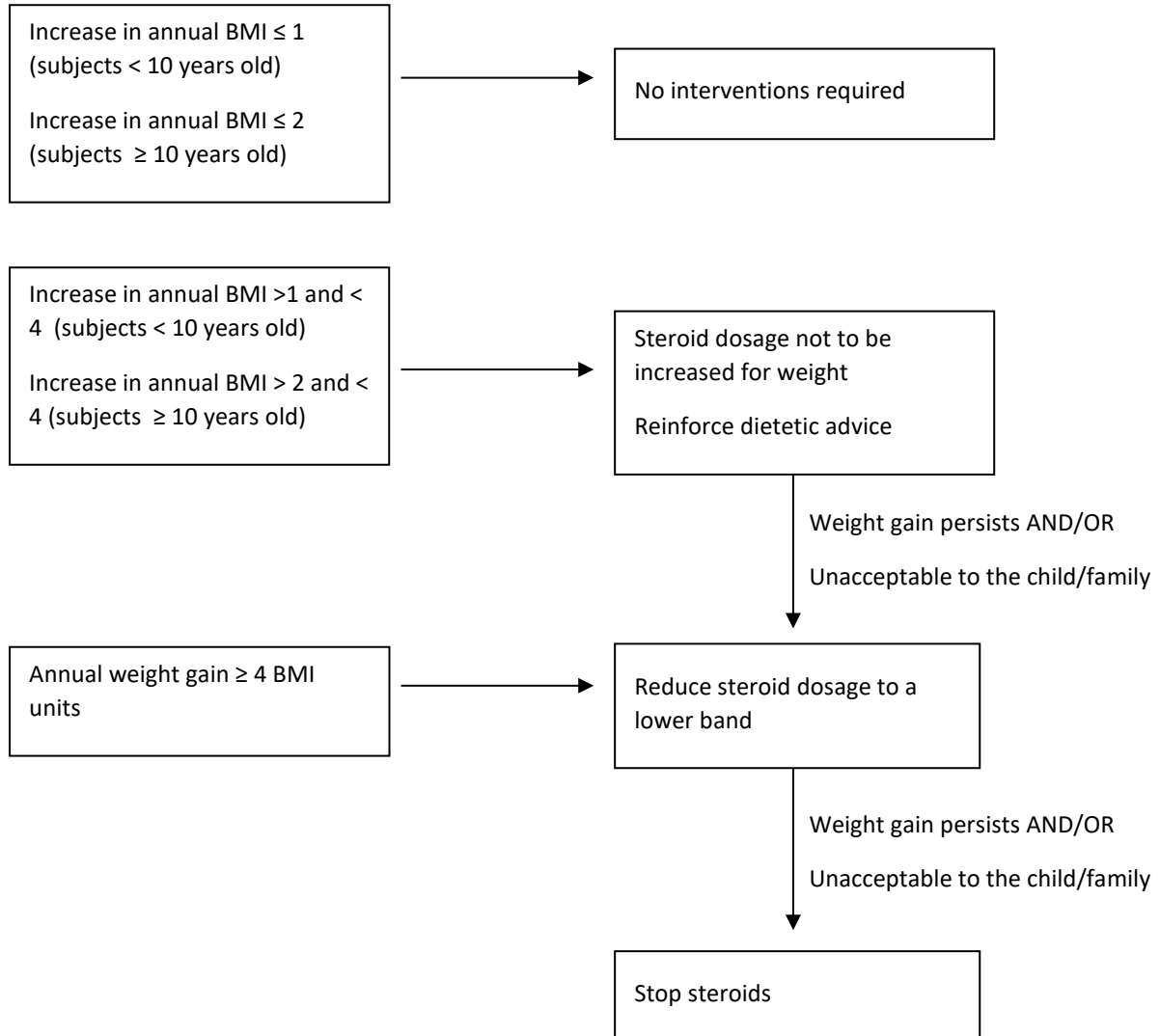
WEIGHT GAIN

Precautionary interventions:

Standardized dietary advice at screening, baseline/entry visit and at all follow up visits.

Assessment:

Weight measurements and BMI calculation at screening, baseline/entry visit at all follow up visits.



MANAGEMENT OF OTHER ADVERSE EVENTS (WHICH DO NOT REQUIRE STUDY DRUG DOSAGE MODIFICATIONS)

