

## Questionnaire related to the Parkinsonism-Dystonia scale for infants and young children

This scale has been developed to rate parkinsonism in infants and young patients. The features of parkinsonism in this age group are based on the known consequences of congenital dopamine deficiency states in disorders of biogenic amine metabolism. The scale will also be helpful for the evaluation of patients with a broader range of acquired or genetic conditions associated with central hypokinesia.

Given the early stage of development of this age group, patients' active cooperation is not expected and it is necessary that raters are familiar with the stages of motor development to score the presence and severity of motor delay.

As developers of this new scale we request your opinion about this assessment as this is a crucial step in its development. Your response is very much appreciated.

Please, respond to the following questions after application of the PARKINSONISM-DYSTONIA SCALE FOR INFANTS AND YOUNG CHILDREN to your patients

	YES	NO
1. Do you find the scale relevant?	<input type="checkbox"/>	<input type="checkbox"/>
2. Do you think that the scale addresses all non-motor aspects of dopamine deficiency states in infants and young children	<input type="checkbox"/>	<input type="checkbox"/>
If "NO", please provide information about the gaps in its content:		
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3. Do you think that the scale addresses all the motor aspects of dopamine deficiency states in infants and young children.	<input type="checkbox"/>	<input type="checkbox"/>
If "NO", please provide information about the gaps in its content:		
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4. Does this scale help you to better assess the status of infants and young children with parkinsonism?	<input type="checkbox"/>	<input type="checkbox"/>

5. Do you think this scale is comprehensive?

 

If "NO", please provide information about the gaps in its content:

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6. Do you think this scale is too long?

 

7. Do you find the questions easy to understand?

 

If "NO", please provide comments about how to simplify:

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8. Did you find any particular question(s) difficult to answer?

 

If "YES", please provide information about the question(s) which were difficult to answer:

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9. Do you have any additional comments or ideas to improve the current version of this scale?

 

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## **Summary of responses from experts to the questionnaire related to development of the parkinsonism-dystonia scale for infants and young children**

The questionnaire was sent to several experts, some of whom were from the same institution; three experts from one institution responded as a group (SJD), two experts from another institution answered separately (SG, VL), and the rest responded individually (EF, MK, JM).

1. **“Do you find the scale relevant?”**: All authors answered favorably
2. **“Do you think that the scale addresses all non-motor aspects of dopamine deficiency states in infants and young children”**: Two experts emphasized the need for greater specificity in categorizing symptoms, particularly in sleep disturbances, gastrointestinal manifestations, and emotional lability, to ensure that important details and relationships are accurately identified. Two other experts stressed the value of structured, standardized approaches to better understand and monitor the developmental and neuropsychological challenges in these patients.
3. **“Do you think that the scale addresses all the motor aspects of dopamine deficiency states in infants and young children”**: One expert answered NO, three answered YES and two provided comments but did not specify an answer. The expert who answered NO suggested modifications for the Oculogyric Crisis section to include clearer duration categories and frequency options. Three authors emphasized the need for greater specificity and clarity in assessing rigidity, dystonia, hypotonia, bradykinesia, hypokinesia, and posture-related issues, specifically:
  - 1) Differentiation between bradykinesia and hypokinesia and the inclusion of an assessment of voice characteristics.
  - 2) Addition of items to explore posture control as well as developmental milestones; with a note that rigidity, hypokinesia, or dyskinesic movements may cause loss of head/trunk control, which could be misattributed to hypotonia.
  - 3) Separation of upper-face dystonia from lower-face/oromandibular dystonia to better capture dystonia-parkinsonism features.
  - 4) The need to distinguish increased tone from true rigidity, and hypotonia from weakness.

Overall, the experts' feedback emphasized the need for clearer distinctions between motor phenomena with improved guidelines for evaluating and categorizing dystonia, hypotonia, and other motor signs, and a more detailed assessment of postural control

4. **“Does this scale help you to better assess the status of infants and young children with parkinsonism?”**: Two experts answered YES, one answered MOSTLY YES, and two provided comments without specifying a direct answer. Four experts emphasized the following points:

- 1) The scale can differentiate between mild TH deficiency, severe AADC deficiency, and undiagnosed cases. It was suggested to score rigidity separately for the upper and lower limbs.
- 2) The importance of distinguishing between bradykinesia and hypokinesia was highlighted. It was recommended to score rigidity limb-by-limb to identify patterns and to clarify whether tremor is paroxysmal or interruptible.
- 3) The absence of information on daily symptom fluctuations was noted, with a suggestion that this may be an important aspect to include.
- 4) It was pointed out that increased dystonia during activity may not reflect severity but could instead indicate fatigue.

The feedback collectively recommended clarifying and refining certain items to better evaluate motor symptom patterns, daily fluctuations, and distinctions between conditions like dystonia and parkinsonism.

5. **“Do you think this scale is comprehensive?”**: Three experts answered YES, one did not answer and another one made a comment suggesting balancing the scale's comprehensiveness and practicality by ensuring all features are equally weighted, as oculogyric crises seemed overemphasized compared to bradykinesia, tremor, and rigidity. They recommend focusing on the most affected area for rigidity scoring to maintain the practicality of the scale, prioritizing functional impact rather than scoring each limb individually
6. **“Do you think this scale is too long?”**: One expert didn't answer, and four answered NO
7. **“Do you find the questions easy to understand?”**: One expert did not answer, three answered YES and one answered MOSTLY YES. The latter found the questions mostly clear but noted confusion about the time frame for rating symptoms like oculogyric crisis and dyskinesia; and suggested rating the maximum severity, frequency, and duration of symptoms over the past month or three months to avoid underestimating the symptom
8. **“Did you find any particular question(s) difficult to answer?”**: One expert did not answer and four answered NO
9. **“Do you have any additional comments or ideas to improve the current version of this scale?”** Four experts provided additional comments and suggestions including:
  - 1) Distinguishing between dyskinesias caused by the disease and those caused by medication.
  - 2) Adding a severity factor based on motor abilities, communication, cognitive functioning, response to therapy, and disease progression.
  - 3) Including minimal diagnostic criteria for clinical definitions of infant and child parkinsonism and parkinsonism-dystonia to aid clinicians in patient selection. It was also suggested considering a video recording protocol as a supportive tool for the scale.

4) Emphasizing the importance of specifying the time period for evaluating symptoms in caregiver questions to assess the patient's current state rather than their previous clinical status.

5) Recommending that the "motor delay" item be placed after the examination section and specifying the acceptable interval for past developmental assessments in treated children. A clarification was also requested on what constitutes "dopaminergic medications" for dyskinesia.