**Table 2.** Summary of study assessments and procedures in the open period

| | Open trial | | | | |
|---|---|---|---|---|---|
| | Day1 | Week 13 | Week 26 | Week39 | Week52 |
| Prescription (NPC-15) | ← | | | | → |
| Melanin index | X | | | | |
| Facial pigmentation | X | | | | X |
| Neurologic severity scale score [b] | | | | | X |
| Hearing test [c] | | | | | X |
| 5-meter walk test [d] | | | | | X |
| Acute skin symptoms | | | | | X |
| Skin cancer | | | | | X |
| Laboratory test for Research (urine)[e] | X | | X | | X |
| Laboratory tests (blood and urine) [f] | | | | | X |
| Secondary sexual | | | | | X |

| | | | | | |
|---|---|---|---|---|---|
| characteristics status [g] | | | | | |
| Adverse events | ← | — | — | — | → |
| drowsiness and dizziness | X | X | X | X | X |
| Body weight | X | X | X | X | |
| Medication status | | X | X | X | X |

[a] The study will be conducted only on patients who are deemed by the principal/sub-investigator at Visit 305 (after 52 weeks of the open study), to require a visit to the hospital for evaluation of adverse events, etc.

[b] Neurologic severity scale score will be evaluated in patients aged 3 years or above.

[c] Methods used for testing hearing (pure-tone audiometry or conditioned play audiometry, etc.) will be recorded in the medical records.

[d] The 5-meter walk test will be conducted including the patients who wear the brace when the principal/participating investigator deems the patient can tolerate the test. The presence or absence of brace or the type of brace used will be recorded in the medical records. When the patient wears the brace, the tests at visit 1 and Visit 305 will be conducted using the same brace as far as possible, and if the brace is changed, the kinds of brace and the reason for the change of brace will be described in the medical records.

[e] Urine examination for research use: Oxidative stress markers (Malondialdehyde,

8-hydroxy-2-deoxyguanosine, Hexanoyl-Lys) and N-acetyl 5-methoxytryptamine metabolites (6-sulfatoxymelatonin).

[f] Urine examination: Urinary protein and urinary urobilinogen.

[g] Confirmation of secondary sexual characteristics status and measurement of prolactin levels in blood. To be performed on patients between 10 and 17 years of age.