

Leveraging Rett Syndrome Natural History and Caregiver Input to Assess Treatment Effects in Embolden™ Pivotal Trial of NGN-401

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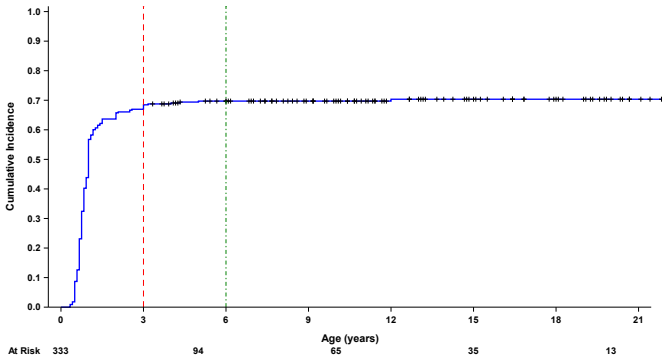
Supplemental Data: Clinically meaningful gains concentrate before age 3 yrs and stay low at age ≥ 3 yrs, with little difference at age ≥ 6 yrs



Fine Motor

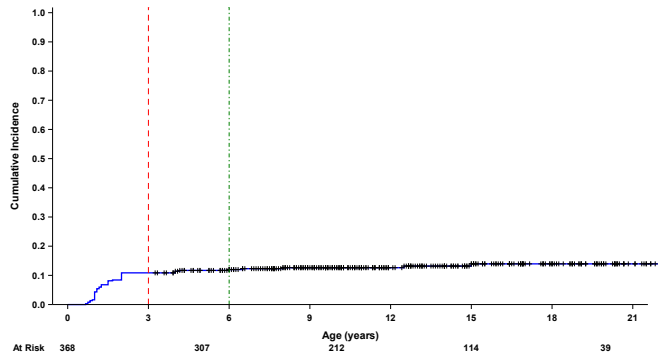
Example Milestone: Used a pincer grasp

% ever gained/regained: **71.4%**
At age ≥ 3 years: **5.3%**
At age ≥ 6 years: **2.0%**



Example Milestone: Used a spoon/fork to eat without assistance

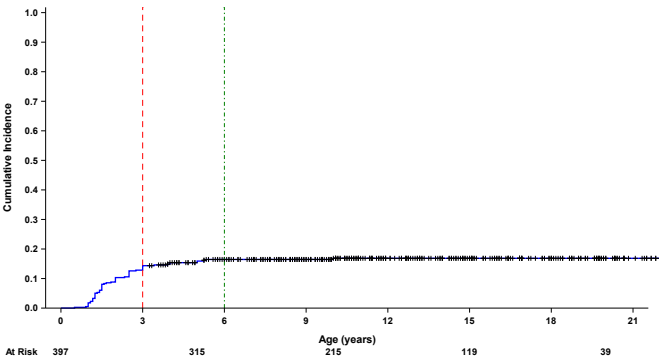
% ever gained/regained: **13.9%**
At age ≥ 3 years: **3.1%**
At age ≥ 6 years: **2.2%**



Gross Motor

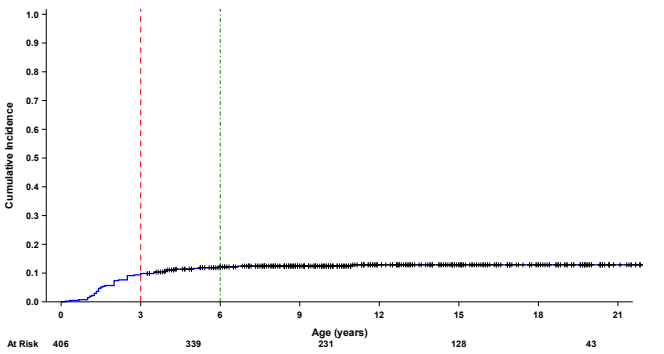
Example Milestone: Climb up stairs without help

% ever gained/regained: **17.3%**
At age ≥ 3 years: **4.5%**
At age ≥ 6 years: **0.4%**



Example Milestone: Climb down stairs without help

% ever gained/regained: **12.9%**
At age ≥ 3 years: **3.5%**
At age ≥ 6 years: **1.0%**



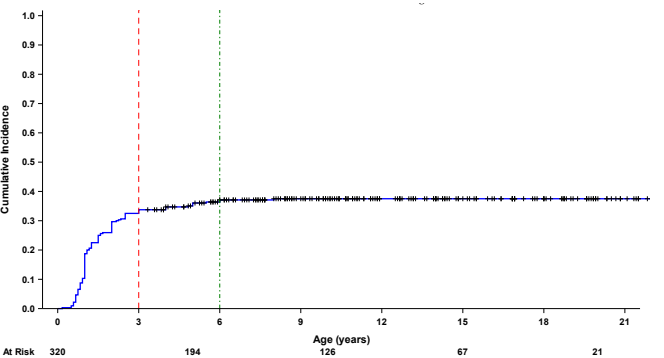
Age at which caregiver reports milestone was first gained; if age gained is unknown, age at which caregiver reports milestone was regained



Communication

Example Milestone: Followed a command without a gesture

% ever gained/regained: **38.3%**
At age ≥ 3 years: **6.1%**
At age ≥ 6 years: **1.5%**



Example Milestone: Waved bye bye

% ever gained/regained: **46.9%**
At age ≥ 3 years: **2.1%**
At age ≥ 6 years: **0.3%**

