

What Is the Starting Dose(s) of Selexipag in Patients Aged ≥ 2 to < 18 Years With Pulmonary Arterial Hypertension to Achieve Exposure Comparable With Adults?

STUDY DESIGN

• Prospective, multicenter, open-label, single-arm phase 2 study:

- **Cohort 1:** aged ≥ 12 to < 18 years (N = 22)
- **Cohort 2:** aged ≥ 6 to < 12 years (N = 21)
- **Cohort 3:** aged ≥ 2 to < 6 years (N = 20)



• Given weight-based starting doses of selexipag:

- **200 μg bid:** ≥ 50 kg
- **150 μg bid:** ≥ 25 to < 50 kg
- **100 μg bid:** ≥ 9 to < 25 kg



Titration over 12 weeks

Outcome: Safety, tolerability, and exploratory efficacy

RESULTS

Stratifier	Value	No.	Estimated $AUC_{\tau,ss,combined}$ (90% CI)	Expected $AUC_{\tau,ss,combined}$ (90% CI)	Ratio (90% CI)
Cohort 1	≥ 12 to < 18 y	21	21.56 (17.32-26.84)	21.47 (19.26-23.94)	1.00 (0.82-1.23)
Cohort 2	≥ 6 to < 12 y	21	20.40 (16.83-24.73)	19.14 (16.92-21.63)	1.07 (0.91-1.24)
Cohort 3	≥ 2 to < 6 y	17	18.57 (15.16-22.74)	15.06 (13.40-16.92)	1.23 (1.04-1.47)
Starting dose (body weight group)	200 μg bid (> 50 kg)	12	22.48 (16.77-30.14)	21.19 (18.28-24.56)	1.06 (0.81-1.39)
	150 μg bid (≥ 25 to < 50 kg)	19	20.95 (17.15-25.58)	20.92 (18.37-23.82)	1.00 (0.82-1.22)
	100 μg bid (≥ 9 to < 25 kg)	28	18.92 (15.97-22.42)	16.25 (14.76-17.90)	1.16 (1.02-1.33)
Total	Not applicable	59	20.25 (18.00-22.78)	18.61 (17.29-20.02)	1.09 (0.98-1.21)

- The weight-based dosing regimen had **similar exposure in children** to adults (as indicated by the **AUC estimated/expected ratios**)
- 62/63 patients experienced ≥ 1 adverse event and 33/63 patients reported serious adverse events
 - 46.0% vomiting
 - 39.7% headache
 - 36.5% diarrhea
- 11 deaths reported, none related to selexipag

In this study, using a weight-based dosing regimen of selexipag in patients aged ≥ 2 to < 18 years with pulmonary arterial hypertension reached similar exposure to adults and was well tolerated.