

A new way to look at Phe control

The Sustained Phe Response (SPR) model uses non-parametric regression to estimate expected Phe levels based on known levels^{1,2}

SPR is a robust and conservative estimate to define durable response and characterize Phe response profiles in both individuals and cohorts¹⁻³

1

A “smoother” (a locally weighted moving average) is applied to each patient’s longitudinal blood Phe profile. Smoothed Phe over time optimally minimizes error and allows estimation of expected values at any time point

3

SPR is achieved when the upper bound of the confidence band crosses below commonly used blood Phe level thresholds (SPR_p ≤ 360 in this example)

5

SPR_p is the proportion of follow-up time that the confidence band remains below the given threshold*, helping to demonstrate the durability of blood Phe response over time (in this example, SPR_p ≤ 360 = ~99%)

2

The 95% confidence band represents the range of plausible expected Phe values at any time point

- Wider when measures are sparse or variable
- Narrower when measures are consistent

4

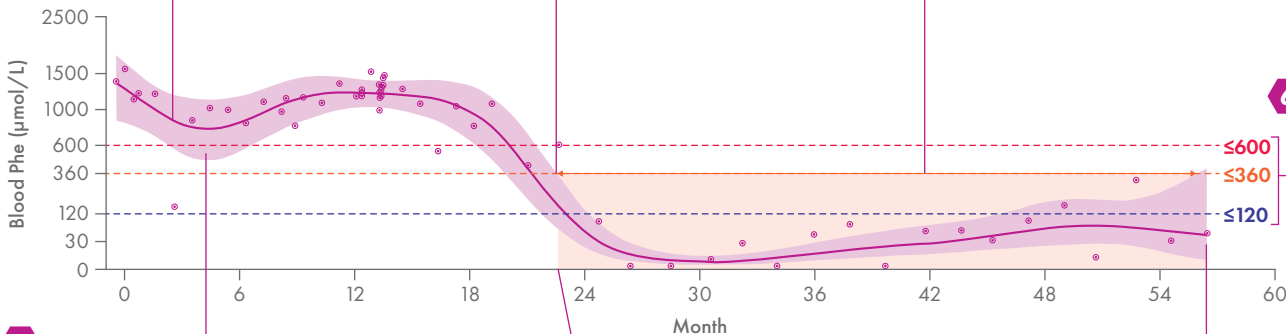
SPR_i is the initial time point at which SPR is achieved (in this example, SPR_i ≤ 360 = ~22 months)

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Limitations include “end splay”[†] and a retrospective methodology[‡]

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SPR parameters can be evaluated using any predefined Phe threshold



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* SPR_p is calculated as the percentage of follow-up time that the individual sustained the response for, starting from when SPR was initially achieved through the end of the follow-up period^{1,2};

[†]End splay is when SPR appears to be lost at the final measurement because there are no additional data to ensure its continuation^{1,3};

[‡]The SPR model cannot be used to predict the next blood Phe level due to its retrospective nature. Results should be interpreted with caution^{1,3}

Phe, phenylalanine; SPR, sustained Phe response; SPR_i, initial sustained Phe response; SPR_p, sustained Phe response proportion

1. Harding CO et al. *Genet Med* 2025;27:101459; 2. Harding CO et al. *Mol Genet Metab Rep* 2024;39:101084; 3. BioMarin data on file

