



From the Editor

It is easy to understand that people are excited about **Kuvan**, but I feel a large responsibility to help paint a realistic picture. I hope that I am doing that through the two editorials on Kuvan (this issue and in the Fall 07 issue). Not everyone will agree with these points of view. I welcome further discussion from readers.

After I read a draft of the editorial in this issue, I decided to go to the FDA website (www.fda.gov/cder/foi/nda/2007/022181TOC.htm) and review the documents on Kuvan's approval for myself (hundreds of pages). Several things really jumped out at me. First, the FDA unequivocally rejected (in many places throughout the document) the idea that Kuvan should be used for any purpose other than "reducing blood phe levels" (presumably it is only for those who are *not* well controlled on diet). **So if anyone is trying to sell you on the idea that Kuvan is there to help you liberalize the diet, they are incorrectly interpreting the FDA's firm conclusions based on the data presented to them.** Read the report for yourself. While there is much technical data and analysis presented, there also is discussion in parts of the report that anyone can understand.

Second, I was struck by how many studies the FDA is requiring as "post-marketing commitments" because these issues were not dealt with in the clinical trials performed by BioMarin (with prior FDA approval). In the FDA report, among "specific deficiencies noted" were:

- ▶ Studies did not enroll patients younger than age 4.
- ▶ Duration of exposure was inadequate to make an assessment of long-term safety.
- ▶ No studies incorporated neurocognitive outcomes.
- ▶ No studies assessed growth and development.

We have to wonder why the drug was approved at this time, especially why it was "fast tracked" through the approval process, given the many studies that must still be conducted over a period of years in order to prove both long-term efficacy and safety, particularly for children.

As I have said before, **everyone needs to weigh the risks versus benefits of using the drug for their unique situation.** I hope that in this issue I have given you more information to consider in making such an important decision.

*Virginia Schuett, Editor
6869 Woodlawn Ave. NE #116
Seattle, WA 98115
e-mail: schuett@pkunews.org
website: www.pkunews.org*