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**Author:** Bellanti, Francesco  
**Title:** From data to models: reducing uncertainty in benefit-risk assessment: application to chronic iron overload in children  
**Issue Date:** 2015-09-24
Propositions to the doctoral thesis

“From data to models: reducing uncertainty in benefit-risk assessment.
Application to chronic iron overload in children”

1. PKPD modelling has been always used in the singular; assessment of benefit-risk balance imposes its use in the plural (this thesis)
2. Evidence generation can be complemented by evidence synthesis to achieve a more comprehensive understanding of the benefit-risk balance of a paediatric medicine (this thesis)
3. The exposure of deferiprone in children younger than 6 years of age is comparable to adults receiving similar dose levels in mg/kg (this thesis)
4. The time required to reach clinical response in chronic iron overload is determined by baseline serum ferritin levels and most importantly by compliance to chelation therapy (this thesis)
5. The low frequency of the adverse events, which often pertains to the most concerning ones, is not a limitation for a model-based approach (this thesis)
6. One of the hardest ethical challenges of paediatric research is the balancing of benefit from a study against the harm and risks (Sammons et al. Paediatrics and Child Health; 2012; 22 (2): 47-50).
7. Models can be used to drive inference minimizing the number of individuals and samples needed in clinical trials (Manolis et al. Paediatric anaesthesia; 2011; 21 (3): 214-21).
9. When treatment is not working, ask: non-responder or non-adherer? (B. Vrijens 2009)
10. We often fail to allow for the possibility that evidence that should be critical to our judgment is missing – what you see is all there is (WYSIATI) (Tversky et al. Science; 1974; 185: 1124-1131)
11. Formulas do not suffer from “interpretation”. Given the same input they always return the same answer. (Adapted from “Thinking, fast and slow”; D. Kahneman)
12. It is not only for what we do that we are held responsible, but also for what we do not do (Moliere)
13. I have been impressed with the urgency of doing. Knowing is not enough; we must apply. Being willing is not enough; we must do (Leonardo da Vinci)
14. Learning never exhausts the mind (Leonardo da Vinci)

F. Bellanti
Leiden, 24 September 2015