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Treosulfan pharmacokinetics and dynamics in pediatric allogeneic stem cell transplantation

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Stellingen behorende bij het proefschrift

Treosulfan pharmacokinetics and dynamics in pediatric allogeneic stem cell transplantation

1. Body surface area-based dosing of treosulfan leads to an overestimated dose resulting in higher exposure in neonates and infants. – *this thesis*
2. Treosulfan exposure is not associated with clinical outcome, but therapeutic drug monitoring of treosulfan is useful to prevent acute toxicity. – *this thesis*
3. Text mining tools are very effective in detecting suspected adverse events in electronic health records. – *this thesis*
4. Because of its favorable gonadal toxicity profile and lack of need for TDM, treosulfan is preferred over busulfan in the conditioning regimen for non-malignant diseases. – *this thesis*
5. When it comes to dosing of drugs we should consider children as small adults and neonates and infants as immature children. – *BJ Anderson and NH Holford, Arch Dis Child, 2013. 98(9):737-44*
6. Therapeutic drug monitoring is a helpful tool to optimize outcome, but should not be a goal in itself.
7. It is naïve to believe that the complex relationship between disease and disease status, type of donor and cell source used after giving multiagent chemotherapy combined with serotherapy will have a simple relationship to even complex descriptors of any one of the conditioning agents used. – *Ben Hassine et al., Front Pediatr, 2021. 9:775485*
8. Stem cell gene therapy is a good alternative for allogeneic stem cell transplantation in some non-malignant inherited diseases, however its access is challenged by complex market access due to extreme high pricing.
9. It would be possible to describe everything scientifically, but it would make no sense; it would be without meaning, as if you described a Beethoven symphony as a variation of wave pressure. – *Albert Einstein - We can try to explain everything scientifically, but sometimes we just have to sit back and admire the beauty of things.*
10. The idea of growth and comfort do not co-exist. If you are going to grow, you will be uncomfortable. – *Ginni Rometty, former CEO of IBM – Everyone experiences difficult times in life, but it is in these moments you will experience growth.*