Abstract of thesis

The majority of medicinal products have not been investigated in the paediatric population although they are used for treatment in this population. This may constitute a significant health threat because medicinal products may have a potentially different safety and efficacy profile in this population as compared to adults. In response to this, the EU and the US were the first regulatory authorities to implement mandatory paediatric legislation to aid paediatric drug development. They have most often been considered successful based on evaluations in the individual regions, but investigations that compare and evaluate the impact of the differences in the paediatric framework across regulatory jurisdictions remain unexplored.

This thesis provides insight into the impact of EU and US paediatric regulations of promoting the availability of medicines for the paediatric population, especially in a transatlantic context. On this basis, two retrospective cross-sectional studies investigate and compare the requirements for paediatric studies as agreed by the regulatory authorities for approved innovative medicines and examine the possible output by comparing the guidance for use in the prescription information. In addition, two case studies are performed: one investigating the progress of agreed Paediatric Investigation Plans (PIPs) as mandated through the EU Paediatric Regulation, the other exploring the contribution of agreed PIPs to the development of Coronavirus Disease 2019 (COVID-19) vaccines for use in the paediatric population.

The studies found that paediatric development plans were more often mandated in the EU than in the US based on the differences in the paediatric legislation when comparing similar indications granted for innovative medicinal products approved in both regions. However, in a comparable study sample, the guidance for paediatric use was similar after approximately five years of follow-up from the marketing authorisation. For the PIPs agreed to by the EMA in 2011, a relatively low completion rate was observed, and a proportion of PIPs being evaluated were inactive, with a low prospect of completion. Further, for COVID-19, a condition possibly affecting the entire paediatric population and with a strong societal need, the PIPs might not be a contributing factor for adolescents and children; however, they seemed to ensure that the development covered the needs of the entire paediatric population.

This thesis concludes that differences in mandated paediatric drug development exist between the EU and the US, but this is not reflected in the prescribing information after a median of five years from the approval of the medicinal products.