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Pediatric drug development in rare and orphan diseases

Dinah Duarte

National Authority of Medicines and Health Products, Portugal

Learning Objectives: Discuss the strategic aspects, experiences and learnings with regard to regulatory framework for pediatric orphan drug. Share the experience from paediatric development process of medicines and review the opportunities offered by the different legislations for treatment of rare diseases; discuss the regulatory aspects that may impact approval of pediatric orphan drugs

Proposal Details: Drug development is no longer possible without considering children and the orphan indication and/or rare disease pediatric setting involves unique considerations in addition to the more common challenges of clinical research and therefore poses extra challenges in conducting a research study. The medicinal products for paediatric use pose a challenge due to the heterogeneity of the considered age groups and the impossibility of extrapolating clinical results between adults and children. These difficulties could impact in children's access to medicines innovation. European legislation in medicines has been in line with the path of the United States Food and Drug Administration with new procedures for granting marketing authorization now include accelerated and conditional approvals, leading to quicker access of new drugs to patients. In this evolving scenario, guidelines on the evaluation of medicinal products are subject to continuous revision. We intend to present the current status and forthcoming activities related to principles behind successful development of paediatric orphan drugs, focusing on development challenges and critical needs and provide information on various strategies designed to overcome these challenges. We will present recent examples of the pediatric orphan drug application process from designation to approval (orphan market exclusivity, similarity and significant benefit) and future developments such as the introduction of rare paediatric diseases designations and potential implications for orphan drugs.

dinah.duarte@infarmed.pt

Anti-ageing dietary supplements and nanocosmeceutical products newly developed from Thai grape seed extracts

Prapaipat Klungsunya

Thailand Institute of Scientific and Technological Research, Thailand

Ageing is a natural process which is accompanied by normal physical, chemical, and biological changes in the body. The world population is rapidly ageing. By 2050, the number of the people aged 60 and older will rise to about 22%. Therefore, anti-ageing products are of great demand for consumers of this group. Our latest study was carried to search and develop new anti-ageing products containing natural anti-oxidant substances from seeds of grape *Vitis vinifera* cv. Ribier grown in Thailand. It has been known that oligomeric proanthocyanidins (OPCs) are the most abundant of flavonoids found in the grape seeds, especially the red grapes (*Vitis vinifera*). A large number of publications have proven that OPCs possess the powerful anti-oxidant property and that have been suggested to reduce the risk of many oxidative-mediated diseases including cardiovascular disease, Alzheimer's disease and cancers. The extensive research both *in vitro* and *in vivo* studies shown OPCs can act as anti-carcinogenic agent by activities include reduced proliferation, increased apoptosis, cell cycle arrest in tumor cells. By 3 year-study, we could prove various pharmacological activities of the OPCs in Thai grape seed extracts including DNA-protective activity against UV and free radicals, enzymatic and non-enzymatic anti-oxidant property, anti-mutagenic and anti-inflammatory activities. Following safety determinations at cellular level, Thai OPCs was used as a health promotor in development of dietary supplement capsules. Also, for a beauty reason, it was used as natural active ingredient in formulation of two nanocosmeceutical products including anti-ageing day- and night creams. These products successfully passed the clinical studies and are ready for the commercialization.

prapaipatk@yahoo.co.uk