Abstract. Drug treatment of children is today less regularly based on formal clinical testing than adults. This has led to concerns regarding the safety and efficacy of pediatric medicines and resulted in public action in the United States and the European Union. The reasons for the increasing awareness include better understanding of child physiology, increased trust in GCP (good clinical practice), improved treatment of several severe childhood diseases, a changed view of the child as a subject in society, and more. The US has successfully introduced pediatric legislation that facilitates participation of children in phar-
maceutical innovation, and comparable approaches are now being discussed in Europe and Japan. While the outcome of the EU pediatric regulation in the near future is still open, the US pediatric legislation has been highly successful over the past 8 years and will be revised before it expires in September 2007. Innovative drugs are today being developed by global pharmaceutical companies. Adding pediatric aspects to this development process is a complex task where companies need to build up internal competency. Bureaucratic procedures that could be harmful to the companies’ economic fundaments need to be avoided, and an appropriate ethical framework is required. This needs to be addressed by all partners in healthcare, including regulatory authorities, the pharmaceutical industry, pediatricians, patients and others in a sense of shared responsibility.

9.1 Clinical Drug Development is a Young Discipline

Clinical testing of new medications is historically quite new. At the beginning of the twentieth century it was legal in the United States to claim therapeutic efficacy for any concoction, including pseudo-medicines that promised to cure cancer, arthritis, or tuberculosis. If the product was simply ineffective, it “only” prevented the consumer from purchasing effective treatment. However, often these so-called wonderdrugs were dangerous and could have severe side effects, including permanent disability or death (Hilts 2003). It took several major tragedies, specifically the deaths following the sulfanilamide elixir disaster in 1937 (Wax 1995) or the thousands of children with phocomelia in 1960–1962 following intake of thalidomide by their mothers (Taussig 1962) to mobilize the public opinion sufficiently to introduce requirements, step by step, that drugs needed proof of safety and efficacy (Hilts 2003). Good clinical practice (World Medical Association 2004; International Conference of Harmonisation 1996) has evolved over the ensuing decades as a framework that regulates the performance of clinical testing in humans.

9.2 Off-Patent Use of Medicines in Children

When proof of safety and efficacy by clinical testing became mandatory, clinical trials were conducted in adults, usually in healthy male volunteers. To avoid being sued, drug producers explicitly stated that the product had not been tested in children and therefore could not