

Table S1. Phases of clinical trials for the combined pediatric and adult trials reviewed under BPCA and/or PREA and the Orphan Drug Act.

Type of Drug Reviews	Type of Products	Phases of Clinical Trials				
		1	2	3	4	Unknown
BPCA and/or PREA	Drug	2.2% (1/46) ^a	8.7% (4/46) ^a	71.7% (33/46)	n/a ^b	19.6% (9/46)
	Biologic	n/a ^b	12.5% (1/8)	75% (6/8)	n/a ^b	12.5% (1/8)
Orphan	Drug	8.5% (4/47) ^a	23.4% (11/47) ^a	48.9% (23/47) ^a	2.1% (1/47)	27.7% (13/47)
	Biologic	18.2% (10/55) ^a	32.7% (18/55) ^a	41.8% (23/55) ^a	n/a ^b	20% (11/55)

^aSome overlap between phases of clinical trials

^bn/a – not applicable