Table 1. Classification of extrapolation by paediatric clinical studies

・ Two or more adequate, well-controlled, efficacy and safety trials plus PK data	No extrapolation
・ Single, adequate, well-controlled, efficacy and safety trial (powered for efficacy) plus PK data, ・ Single, controlled or uncontrolled, efficacy and safety trial (qualitative data) plus PK data, or ・ Single exposure-response trial (not powered for efficacy) plus PK and safety data, PK/PD and uncontrolled efficacy plus safety data, or PK/PD plus safety data	Partial extrapolation
・ PK and safety data, or ・ Safety data only	Complete extrapolation

Table 2. Characteristics of drugs approved for paediatric indication (2015 to 2021)

	2015-2021	**2015**	**2016**	**2017**	**2018**	**2019**	**2020**	**2021**
Number of products, n	182	35	21	22	20	28	23	33
Orphan drug designation, n (%)	61 (34)	7 (20)	6 (29)	7 (32)	8 (40)	13 (46)	7 (30)	13 (39)
Development request, n (%)	42 (23)	18 (51)	6 (29)	3 (14)	7 (35)	3 (11)	2 (9)	3 (9)
Approved age range for paediatric use, n (%)								
Newborn infants (0~27 days)	38 (21)	7 (20)	2 (10)	6 (27)	2 (10)	4 (14)	4 (17)	13 (39)
Infants and toddlers (28 days-23 months)	75 (41)	16 (46)	8 (38)	10 (45)	6 (30)	8 (29)	8 (35)	19 (58)
Children (2~11 years)	147 (81)	29 (83)	17 (81)	20 (91)	14 (70)	23 (82)	17 (74)	27 (82)
Adolescents (12~15 years)	182 (100)	35 (100)	21 (100)	22 (100)	20 (100)	28 (100)	23 (100)	33 (100)
Route of administration, n (%)								
Injection	95 (52)	15 (43)	8 (38)	10 (45)	14 (70)	14 (50)	11 (48)	23 (70)
Oral	68 (37)	16 (46)	11 (52)	11 (50)	6 (30)	10 (36)	7 (30)	7 (21)
Other (eye drop, nasal drop, etc)	19 (10)	4 (11)	2 (10)	1 (5)	0	4 (14)	5 (22)	3 (9)
Therapeutic group (ATC code), n (%)								
A: Alimentary tract and metabolism	19 (10)	3 (9)	2 (10)	1 (5)	2 (10)	3 (11)	2 (9)	6 (18)
B: Blood and blood forming organs	18 (10)	2 (6)	2 (10)	3 (14)	4 (20)	2 (7)	4 (17)	1 (3)
C: Cardiovascular system	9 (5)	3 (9)	2 (10)	1 (5)	1 (5)	0	1 (4)	1 (3)
J: Anti-infectives for systemic use	35 (19)	5 (14)	6 (29)	2 (9)	2 (10)	9 (32)	5 (22)	6 (18)
L: Antineoplastic and immunomodulating agents	31 (17)	3 (9)	1 (5)	5 (23)	5 (25)	6 (21)	3 (13)	8 (24)
N: Nervous system	21 (12)	7 (20)	3 (14)	2 (9)	4 (20)	2 (7)	2 (9)	1 (3)
R: Respiratory system	8 (4)	2 (6)	2 (10)	1 (5)	0	2 (7)	1 (4)	0
Other	41 (23)	10 (29)	3 (14)	7 (32)	2 (10)	4 (14)	5 (22)	10 (30)
Availability of adult data at the time of paediatric approval, n (%)								
Paediatric only (no indication for adults)	30 (16)	7 (20)	5 (24)	6 (27)	3 (15)	5 (18)	2 (9)	2 (6)
Simultaneous development in adults and children	112 (62)	19 (54)	13 (62)	9 (41)	15 (75)	18 (64)	15 (65)	23 (70)
Additional indication for paediatric	40 (22)	9 (26)	3 (14)	7 (35)	2 (10)	5 (18)	6 (26)	8 (24)
Approved only for adults, n	622	78	88	78	89	90	94	105
Adult-specific disease, n (%)		3 (4)	3 (3)	0	2 (2)	3 (3)	2 (2)	7 (7)

Table 3. Ages of approval for paediatric indication (2015 to 2021)

	All	Paediatrics only	Adult Paediatric Simultaneous	Addition of children
Number of products, n	182	30	112	40
Newborn infants (0~27 days)	38 (21)	8 (27)	26 (23)	4 (10)
Infants and toddlers (28 days~23 months)	75 (41)	16 (53)	45 (40)	14 (35)
Children (2~11 years)	147 (81)	30 (100)	79 (71)	38 (95)
Adolescents (12~15 years)	182 (100)	30 (100)	112 (100)	40 (100)

Table 4. Characteristics of pivotal paediatric studies for the new drug application (2015 to 2021)

	All	Paediatrics only	Adult Paediatric Simultaneous	Addition of children
Number of products	182	30	112	40
Pivotal trial	162	30	93	40
Randomization, n (%)				
Yes	90 (55)	21 (70)	47 (51)	22 (55)
No	73 (45)	9 (30)	46 (50)	18 (45)
Blinding, n (%)				
Double- blind	71 (44)	15 (50)	39 (42)	17 (43)
Single- blind	5 (3)	2 (7)	3 (3)	0
None (open label)	87 (53)	13 (43)	51 (55)	23 (58)
Comparator, n (%)				
Placebo	59 (36)	12 (40)	33 (36)	14 (35)
Active	21 (13)	7 (23)	8 (9)	6 (15)
None	81 (50)	11 (37)	50 (55)	20 (50)
Location where the study was conducted, n (%)				
Global study	44 (27)	4 (13)	30 (32)	10 (25)
Foreign study	35 (22)	6 (20)	18 (19)	11 (28)
Domestic study	84 (52)	20 (67)	45 (48)	19 (48)