Imagining the Future of Pediatric Formulation Development

Trends in Pediatric Formulation and Delivery Systems

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Disclaimer/Disclosure

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- Presenter is a consultant for industry, government and not-for-profit organizations

Imagining the Future

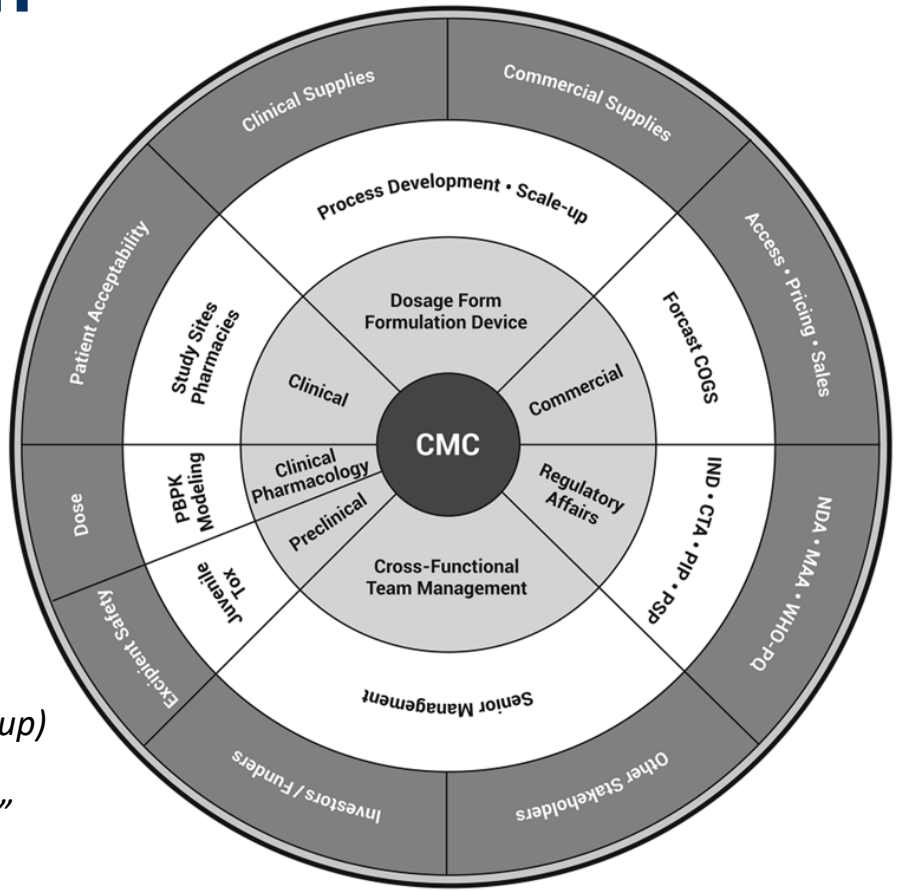
Patient-Centric Approach

- Children!!!
- Parents
- Caregivers
- Healthcare providers
- Payors
- Regulators
- Health authorities
- General public

➢ ***Pediatric Quality Target Product Profile***

CMC-Centric Approach

- Formulator in “driver seat”!
- Partner with clinical pharmacology
  - Progress in modeling and simulation
  - Dose, dosing, age-groups



Walsh et al. Br. J. Clin. Pharmacol. 2021 (C4C Expert Group)

Schaufelberger in “Pediatric Formulations – A Roadmap” Springer; Batchelor & Rose, Eds. 2026

Imagining the Future

Therapeutic/indication focus

Formulation focus

Innovative NMEs & FORMULATIONS

- Small molecules
- Large synthetics
- Biologics: mAb, mRNA
- Biologics: vaccines
- Gene, cell therapies

This Workshop

Next Workshop?

Innovative formulations off-patent products

Baby KJ with two of the researchers who treated him, Kiran Musunuru (left) and Rebecca Ahrens-Nicklas. Children's Hospital of Philadelphia

Young children wait in line to receive dose of chewable mebendazole for intestinal parasites

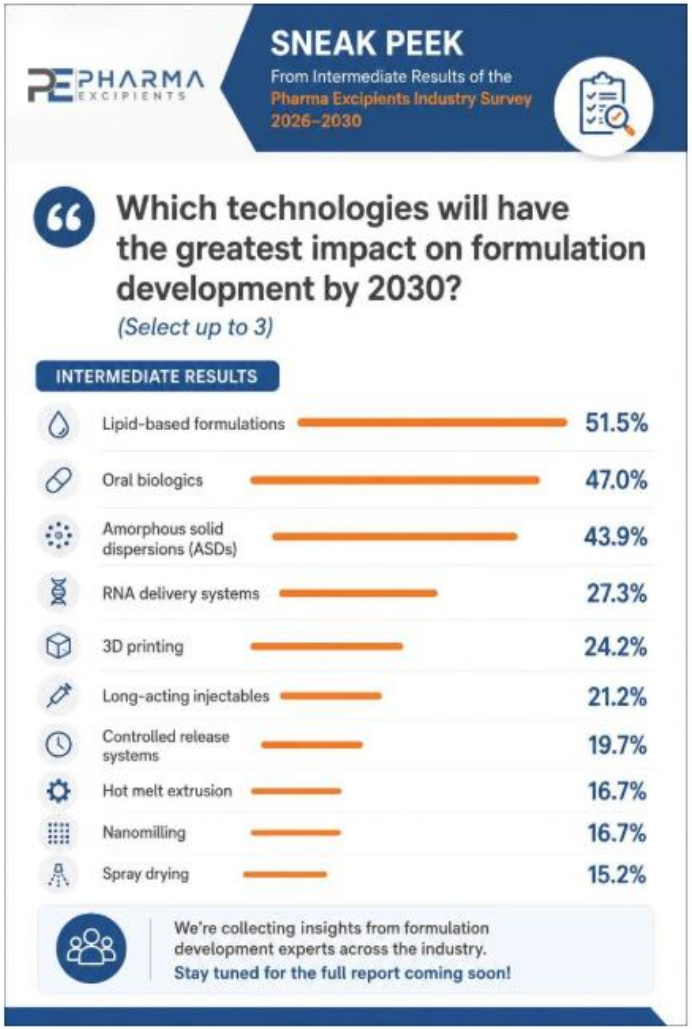
Oral Peptide Delivery – GLP-1 Agonists

- Critical role of excipients!!
- Model for other therapeutic classes?
- Consequences for pediatric formulations?

Excipients

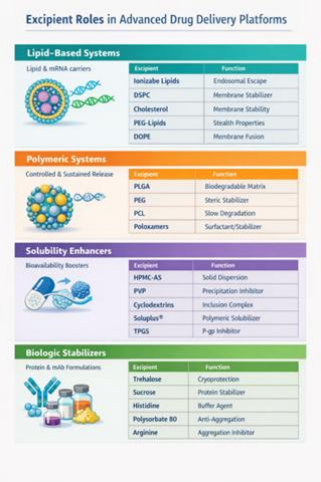
https://www.linkedin.com/pulse/future-pharmaceutical-excipients-20262030-philippe-tschopp-cfsge/

Accessed 12 June 2026



Pivotal Role of Excipients - Vision for Innovation

Inactive ->
Improve ->
Transform



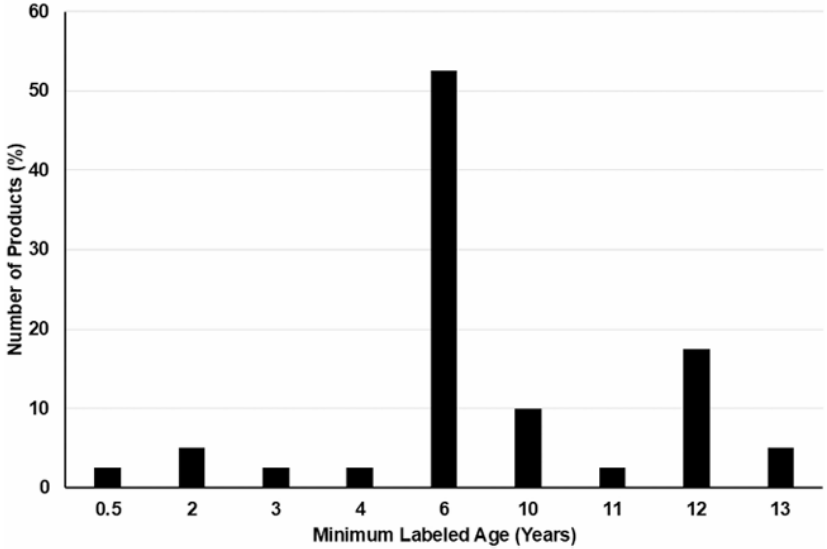
- Improving **bioavailability**
- Delivering increasingly **complex molecules**
- **More effective** and differentiated drug products.
- Enhancing **patient convenience** and adherence
- **Delivery = core strategy**, differentiation, creating intellectual property

Ref: Philippe Tschopp, PE The Excipients Week, Feb 9, 2026
(1) Advanced Drug Delivery Platforms: The Next Frontier in Precision Therapeutics | LinkedIn

FOR PEDIATRICS:

- **Existing Excipients:**
  - > "Accelerate to Zero"
  - > Expand age-ranges
  - > Set boundaries
- **New Excipients**
  - > Enabling, not inhibiting innovation
- **Design of New Excipients**
  - – API and formulation specific
- ***Pre-competitive collaboration & AI supported processes & systems***

Pediatric Oral Extended-Release Products



Pediatric ER formulations: US market landscape

- FDA Pediatric Labeling Changes database (1998-2023)
- N = 42 (16%) of 266 ; > 1/3 for ADHD, > 1/10 for seizure
- 89% minimum age = 6 (or higher)

Ong et al. *J. Pharm Sci* 115 (2026) 104319
https://doi.org/10.1016/j.xphs.2026.104319
hong02@amgen.com (H.J. Ong, Drug Product Technologies, Amgen Inc.)

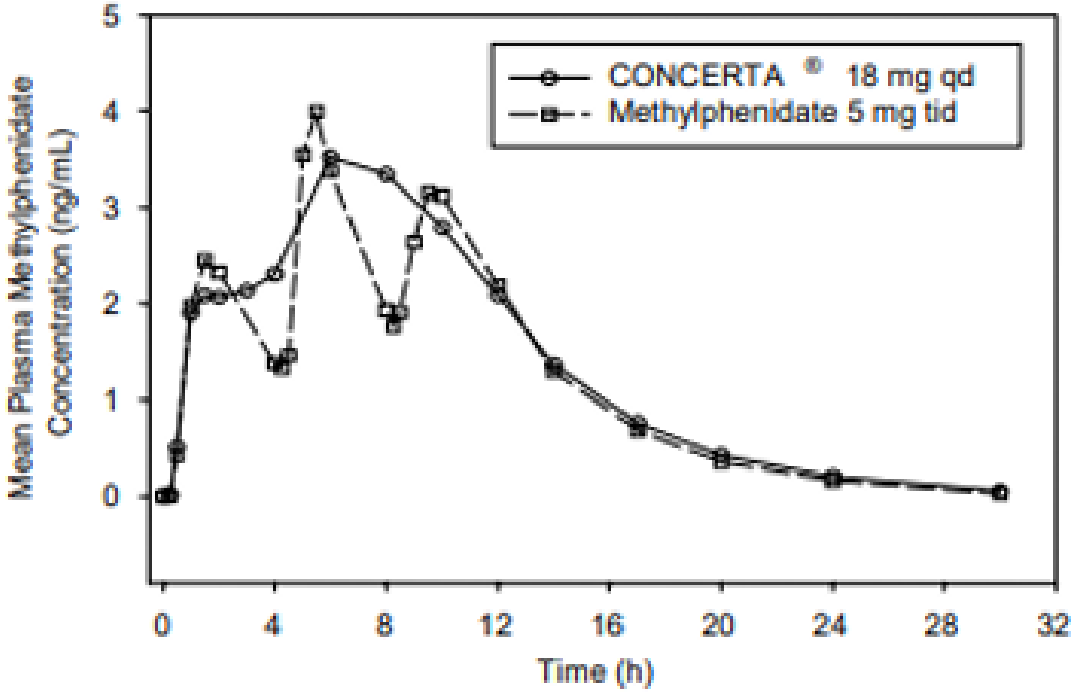
TRENDS

- More pediatric ER products
- Increase in therapeutic scope
- Go below 6 y of age
- Innovation delivery systems, e.g. mini-tablets, gastric retention

CALL for ACTION

- More pre-competitive collaboration
- Pooling resources, sharing data, standardizing procedures

Age-appropriate: School Day



CONCERTA® (methylphenidate hchl)
Prescribing Information (FDA)

ONCE-DAILY
CONCERTA®
methylphenidate HCl
Extended-release tablets 18 mg, 27 mg, 36 mg, 54 mg

“Blood Thinners”: Age-Appropriate Dosing Flexibility

Oral Solid Dosage Form:

Dabigatran etexilate

Oral Pellets 20, 30, 40, 50, 110, or 150 mg/packet

>3 month

Parenteral:

Abelacimab (FXI) “concentrate for dilution” s.c., i.v.

PIP: >1 mo

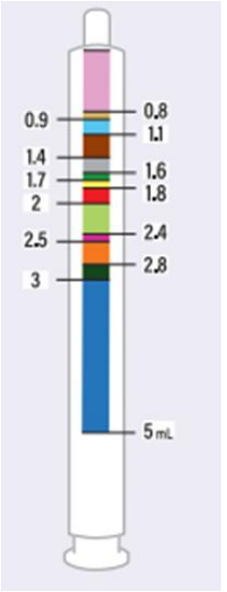
Oral Liquid Dosage Form:

Xarelto® rivaroxaban

Oral Suspension 1 mg/ml

From birth

Inspired by Broselow pediatric emergency tape



Goldenberg et al. 2026 – *J. Thromb. Haemost*. DOI: 10.1016/j.jtha.2026.04.003
“Development of new antithrombotic drugs for children (part1)”

Changing Routes of Administration

Parenteral-to-Oral

- GLP-1 agonists
  - BA enhancers ok for children?

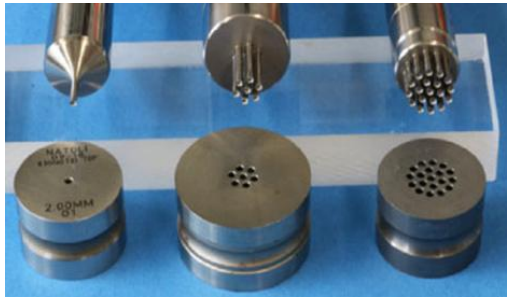
Oral-to-Parenteral

- Anti-retroviral agents
  - e.g., lenacapavir long-acting injectable (6 mo)
- How long is “long”?

➢Consequences for pediatric products?

Emerging Technologies: Minitablets

- 2-3 mm diameter
- ODS or dispersible
- Uncoated, coated
- Sprinkle capsule or stick pack
- Dosing devices?
- Neonates?
- Terminology?



www.ondosis.com
(example)

Taste Masking

AI/ML-based predictions?

REALLY BAD: bitter blockers?

BAD: coating techniques

OK: sweeteners/flavors

Monell Chemical Senses Center www.monell.org

(Re-)Emerging Technologies: Oral Dispersible Films

Klingmann et al. 2020 – *Eur. J. Pharm. Biopharm*. “Acceptability of an orodispersible film compared to syrup in neonates and infants. A randomized controlled trial in neonates and infants”

Purdy et al. 2025 – *AIDS Care*. “Maternal and health care worker preferences for paediatric antiretroviral formulations in neonates exposed to HIV”

Zuplenz® (Ondansetron) 4 mg, 8 mg Oral Soluble Film, MonoSol Rx® Technology; Children ≥ 4 years; Prevention of nausea, vomiting associated with cancer chemotherapy

Emerging Technologies: Needle-free Injection

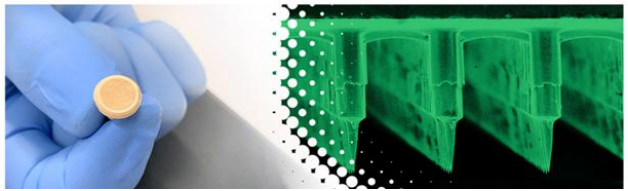
Liquid Jets

2.0 ml high viscosity (50 cP) mAb
Fine jet stream, SC administration
Comparison to needle/syringe SC and IV (pig skin model)

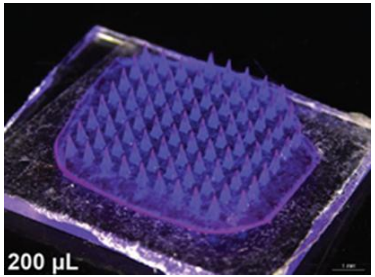
Josowitz, Manoj et al.
Bioeng Transl Med. 2025

PRIME® needle-free platform
www.portalinstruments.com

Microarray Needle Patches (MAP)



www.vaxxas.com – flu vaccine development



"A microneedle vaccine printer for thermostable COVID-19 mRNA vaccines"

Vander Straeten et al. 2023, *Nature Biotechnology*
https://doi.org/10.1038/s41587-023-01774-z

Emerging Technologies: 3D Printing

- Devices, components of delivery systems
- Commercial: SPRITAM® up to 1000 mg (Aprecia Pharmaceuticals)
- Potential for patient-centered delivery, individual dosing

Review article on 3D Printing in Paediatrics

Couti, Iurian et al.
Pharmaceutics 2025

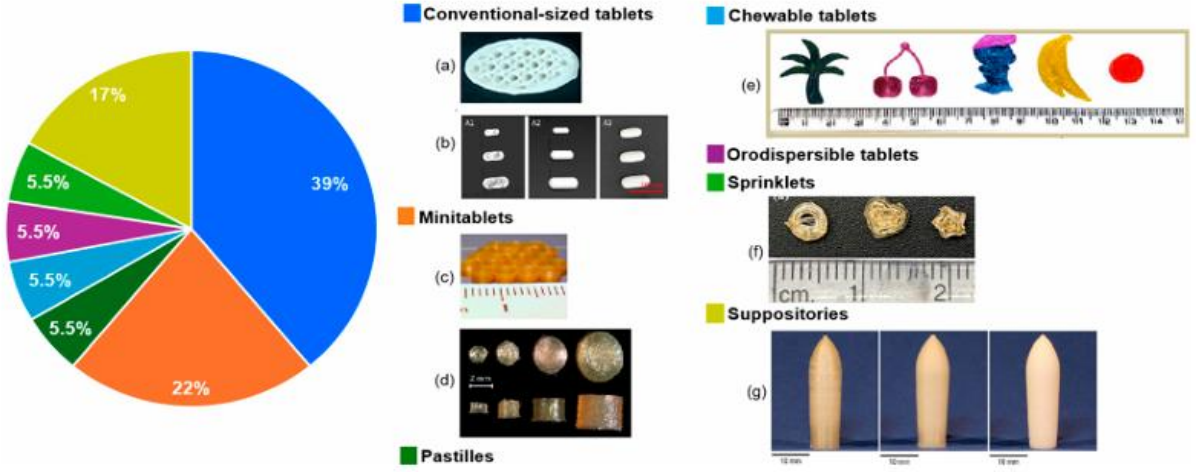
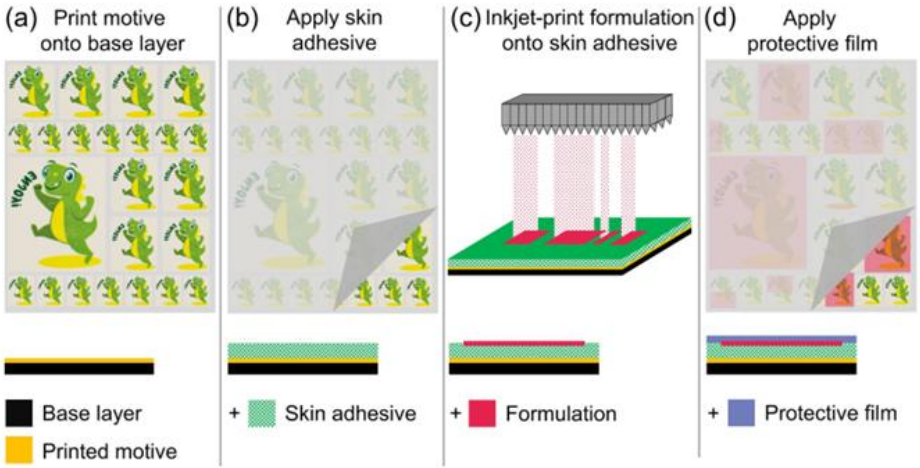


Figure 2. The distribution of papers reporting paediatric medicines manufactured by FDM 3D printing expressed as percentages as a function of dosage forms: conventional-sized (**a**) [39], (**b**) [40]; minitablets (**c**) [41], (**d**) [42]; pastilles; chewable tablets (**e**) [43]; orodispersible films; sprinklets (**f**) [44]; and suppositories (**g**) [45].

Emerging Technologies: Temporary Tattoos?

- Drug-loaded temporary tattoos
- Inkjet-printed; NIR hyperspectral imaging
- Personalized pediatric treatment
- Example: pain treatment, lidocaine, prilocaine



Eder et al.
University Graz/Research Center Pharm. Engineering GmbH
Int. J. Pharm 685 (2025) 126188

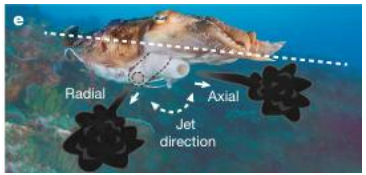
https://doi.org/10.1016/j.ijpharm.2025.126188

Future Technologies?

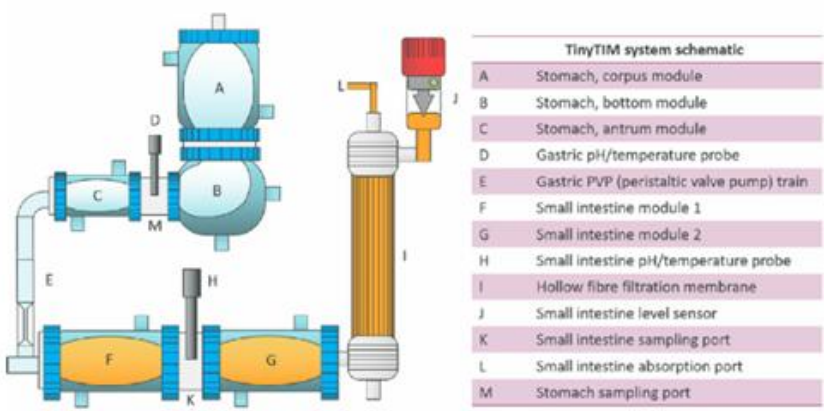
New Delivery?

- Gastric retention (LYNX™)

- Oral BA
  - Learning from nature:
    - > “Sucker fish”
    - > Cattle fish



Prof. G. Traverso – presentation CRS-IC, February 2026
Arrick et al. *Nature* 2024



TinyTIM system schematic	
A	Stomach, corpus module
B	Stomach, bottom module
C	Stomach, antrum module
D	Gastric pH/temperature probe
E	Gastric PVP (peristaltic valve pump) train
F	Small intestine module 1
G	Small intestine module 2
H	Small intestine pH/temperature probe
I	Hollow fibre filtration membrane
J	Small intestine level sensor
K	Small intestine sampling port
L	Small intestine absorption port
M	Stomach sampling port

Lopez et al 2026 *Mol Pharmaceutics*

New Methodology?

- Lab -> organ on a chip -> “baby on a chip”?
- In vitro models, e.g., TIM® InnoGI

Rebirth of Praziquantel – Arpraziquantel - Schistosomiasis

www.pediatricpraziquantelconsoritum.org

Public-Private Partnership (PPP)

Racemic praziquantel -> Pure (R) enantiomer

➔Active enantiomer

➔Reduced bitter taste

Tablet -> Dispersible Tablet

➔Age-appropriate

Commercial Challenges

Challenges:

- Small patient populations
- Difficult to develop
- Not enough incentives?
- Low volume forecasts
- Supply chain: multi-use facilities, # SKUs, obsolescence
- Pricing, reimbursement?

Opportunities:

- Continue PIP & PSP mandates
- Market exclusivity
- Public-Private Partnerships
- Philanthropy -> Technology Platforms -> Commercial viability -> Access

AI Vision Pediatric Formulation Development

Agentic AI?

"Can we bring it all together for kids?"

AI/ML assisted PK modeling

FDA: ELSA 4.0, "HALO"

Virtual Assistants
(for illustration)

FDA IID?

STEP v3?

Virtual assistants?

???

FDA: IID next version?

NIH

STEP v2

EuPFI

Proposed Collaboration EuPFI & NIH (US)

Enhancement AI/ML? PERA ?

"STEP-AI"

STEP

15th Annual Congress EuPFI September 19-21, 2023

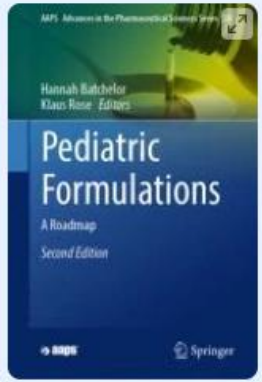
Trends Pediatric Formulation Development

- Towards younger children
- Age-appropriate oral solid dosage forms
- Enhanced oral delivery
- Long-acting injectables
- Needle-free injections
- Central role of excipients
- AI will change everything
- Strong COLLABORATION

Review 2020: Innovative Delivery Systems for Paediatric Medicines – Technology Landscape – UNITAID/WHO 2020

Link: Innovative delivery systems for paediatric medicines: technology landscape

"Pediatric Formulations"
H. Batchelor, K. Rose, Eds.
Springer, 2026
Pediatric Formulations: A Roadmap | Springer Nature Link



YOUR IMAGINATION AT WORK!

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