Mustistakeholder meetings to improve new drug development for rare diseases in the changing regulatory environment

Gilles Vassal, Gustave Roussy

December 12, 2024

ERICA Meeting

European Rare Disease Research Coordination and Support Action

EUROPEAN
COMMISSION

Towards a more science driven and patient centric regulation

Brussels, 26.4.2023
COM(2023) 193 final

2023/0131 (COD)

Proposal for a

REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency, amending Regulation (EC) No 1394/2007 and Regulation (EU) No 536/2014 and repealing Regulation (EC) No 726/2004, Regulation (EC) No 141/2000 and Regulation (EC) No 1901/2006

1. Unmet Medical Needs and High UMN
2. Mechanism of Action PIPs
3. Stepwise PIP

*mechanism of action

CHAPTER VII
PAEDIATRIC MEDICINAL PRODUCTS

EU PROPOSAL

Article 95

European network

1. The Agency shall develop a European network of patient representatives, academics, medicines developers, investigators and centres with expertise in the performance of studies in the paediatric population. = multistakeholder

2. The objectives of the European network shall be, inter alia, to discuss priorities in the clinical development of medicines for children, in particular in areas of unmet medical need, to coordinate studies relating to paediatric medicinal products, to build up the necessary scientific and administrative competences at European level, and to avoid unnecessary duplication of studies and testing in the paediatric population.

Multi-stakeholder collaboration for **prioritisation**

Multi-Stakeholder Meetings

A disease or a class of compounds or devices

Specific objectives:

- Sharing publicly available information and advanced learning
- Define UMN* and update science
- Critical review of ongoing developments and issues to address
- Propose a strategy

Principles:

- ALL relevant stakeholders : clinicians, academics, patients and patient representatives, pharmaceutical companies, regulators, HTA
- Publication in a peer reviewed journal

To facilitate prioritisation

*unmet medical needs

Pediatric Strategy Forum

Setting
- 6 months preparation including TCs with speakers and pharma
- 2 days meeting
- On invitation following expression of interest (pharma, academia, parents)

Output
- Summary on websites
- Article in a peer-reviewed journal

Content
- Biology of disease
- Therapeutic needs including epidemiology, clinical features, standard therapy current needs and future therapeutic plans – Europe and North America
- Insight from patients and parents
- Non-clinical and clinical data on compounds
- Strategic discusssion

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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ACCELERATE
INNOVATION FOR CHILDREN AND ADOLESCENTS WITH CANCER

FDA participation

Paediatric Strategy Forums

Overall more than 300 assets discussed by 2000 participants.

2017

- PSF - 1 **ALK inhibition**
- PSF - 2 **Mature B-cell lymphoma**

2018

- PSF - 3 **CheckPoint Inhibitors**

2019

- PSF - 4 **Acute Myeloid Leukemia**
- PSF Prioritisation **Acute Myeloid Leukemia**

2020

- PSF - 5 **Epigenetic modifiers**
- PSF Prioritisation **BET inhibitors**

2021

- PSF - 6 **Second ALK inhibition**
- PSF - 7 **CAR T cells**
- PSF - 8 **TKI in Sarcomas**

2022

- PSF - 9 **MAPK inhibitors**
- PSF -10 **DNA Damaging agents**

2023

- PSF - 11 **PI3K/AKT/mTOR Pathway**
- PSF - 12 **CDK 4, 6 & 9 inhibitors**

2024

- PSF - 13 **Diffuse Midline Gliomas**
- PSF - 14 **GD2 therapies**

PUBLICATION of PEDIATRIC STRATEGY FORUMS and PRIORITISATION MEETING

N°2 Mature B cell Lymphoma

European Journal of Cancer 110 (2019) 74–85

Available online at www.sciencedirect.com

ELSEVIER

Original Research

ACCELERATE and European Medicine Agency Paediatric Strategy Forum for medicinal product development for mature B-cell malignancies in children

Andrew D.J. Pearson [a,*,1], Nicole Scobie [b], Koenraad Norga [c], Franca Ligas [d], Davy Chiodin [e], Amos Burke [f], Veronique Minard-Colin [g], Peter Adamson [h], Lynley V. Marshall [i,am], Arun Balakumaran [j,2], Bouchra Benettaib [k], Pankaj Bhargava [l], Catherine M. Bollard [m], Ellen Bolotin [n], Simon Bomken [o], Jochen Buechner [p], Birgit Burkhardt [q], Hubert Caron [r], Christopher Copland [s], Pierre Demolis [t], Anton Egorov [u], Mahdi Farhan [v], Gerhard Zugmaier [w], Thomas Gross [x], Danielle Horton-Taylor [y], Wolfram Klapper [z], Giovanni Lesa [d], Robert Marcus [aa], Rodney R. Miles [ab], Kerri Nottage [ac], Lida Pacaud [ad], Rosanna Ricafort [ae], Martin Schrappe [af], Jaroslav Sterba [ag], Remus Vezan [ah], Susan Weiner [ai], Su Young Kim [aj], Gregory Reaman [ak], Gilles Vassal [al]

N°3 Check Point Inhibitors

European Journal of Cancer 127 (2020) 52–66

Available online at www.sciencedirect.com

ELSEVIER journal homepage: www.ejcancer.com

Original Research

ACCELERATE and European Medicines Agency Paediatric Strategy Forum for medicinal product development of checkpoint inhibitors for use in combination therapy in paediatric patients

Andrew D.J. Pearson [a,*], Claudia Rossig [b], Giovanni Lesa [c], Scott J. Diede [d], Susan Weiner [e], John Anderson [f], Juliet Gray [g], Birgit Geoerger [h], Veronique Minard-Colin [h], Lynley V. Marshall [i], Malcolm Smith [j], Paul Sondel [k], Marcis Bajars [l], Claudia Baldazzi [m], Elly Barry [n], Sam Blackman [a], Patricia Blanc [o], Renaud Capdeville [p], Hubert Caron [q], Peter D. Cole [r], Jorge Camarero Jiménez [s], Pierre Demolis [t], Martha Donoghue [u], Mabrouck Elgadi [v], Thomas Gajewski [w], Sara Galluzzo [x], Robert Ilaria Jr [y], Alessandro Jenkner [z], Dominik Karres [c], Mark Kieran [aa], Franca Ligas [c], Israel Lowy [ab], Michael Meyers [ac], Corina Oprea [ad], Vijay G.R. Peddareddigari [ae], Jaroslav Sterba [af], Paul K. Stockman [ag], Peter Suenaert [ah], Uri Tabori [ai], Cornelis van Tilburg [aj], Todd Yancey [ak], Brenda Weigel [al], Koenraad Norga [am], Gregory Reaman [u], Gilles Vassal [h]

N°4 Acute Myeloid Leukemia

European Journal of Cancer 136 (2020) 116–129

Available online at www.sciencedirect.com

ScienceDirect

Original Research

Paediatric Strategy Forum for medicinal product development for acute myeloid leukaemia in children and adolescents☆ ACCELERATE in collaboration with the European Medicines Agency with participation of the Food and Drug Administration

Andrew. D.J. Pearson [a,*,1], C.Michel Zwaan [b,c,1], E.Anders Kolb [d,1], Dominik Karres [e], Julie Guillot [f], Su Young Kim [g], Lynley Marshall [h], Sarah K. Tasian [i], Malcolm Smith [j], Todd Cooper [k], Peter C. Adamson [l], Elly Barry [m], Bouchra Benettaib [n], Florence Binlich [o], Anne Borgman [p], Erica Brivio [b,c], Renaud Capdeville [q], David Delgado [r], Douglas V. Faller [s], Linda Fogelstrand [t], Paula Goodman Fraenkel [u], Henrik Hasle [v], Delphine Heenen [w], Gertjan Kaspers [b,c], Mark Kieran [x], Jan-Henning Klusmann [y], Giovanni Lesa [e], Franca Ligas [e], Silvia Mappa [z], Hesham Mohamed [aa], Andrew Moore [ab], Joan Morris [ac], Kerri Nottage [ad], Dirk Reinhardt [ae], Nicole Scobie [af,ag], Stephen Simko [ah], Thomas Winkler [ai], Koen Norga [aj], Gregory Reaman [ak], Gilles Vassal [a,al]

N°5 Epigenetic Modifiers

European Journal of Cancer 139 (2020) 135–148

Available online at www.sciencedirect.com

ELSEVIER

Review

Paediatric Strategy Forum for medicinal product development of epigenetic modifiers for children ACCELERATE in collaboration with the European Medicines Agency with participation of the Food and Drug Administration

Andrew DJ. Pearson [a,*,1], Kimberly Stegmaier [b,1], Franck Bourdeaut [c,1], Gregory Reaman [d], Delphine Heenen [e], Michael L. Meyers [f], Scott A. Armstrong [b], Patrick Brown [g], Daniel De Carvalho [h], Nada Jabado [i], Lynley Marshall [j], Miguel Rivera [k], Malcolm Smith [l], Peter C. Adamson [m], Amy Barone [d], Christian Baumann [n], Samuel Blackman [o], Vickie Buenger [p], Martha Donoghue [d], Aundrietta D. Duncan [q], Elizabeth Fox [r], Brian Gadbaw [s], Maureen Hattersley [t], Peter Ho [u], Ira Jacobs [v], Michael J. Kelly [w], Mark Kieran [x], Giovanni Lesa [y], Franca Ligas [y], Donna Ludwinski [z], Joe McDonough [aa], Zariana Nikolova [ab], Koen Norga [ac], Adrian Senderowicz [ad], Tilmann Taube [ae], Susan Weiner [af], Dominik Karres [y], Gilles Vassal [ag]

N°6 ALK inhibitors

European Journal of Cancer 157 (2021) 198–213

ELSEVIER journal homepage: www.ejcancer.com

Review

Second Paediatric Strategy Forum for anaplastic lymphoma kinase (ALK) inhibition in paediatric malignancies ACCELERATE in collaboration with the European Medicines Agency with the participation of the Food and Drug Administration

Andrew D.J. Pearson [a,*], Elly Barry [b], Yael P. Mossé [c], Franca Ligas [d], Nick Bird [e], Teresa de Rojas [a], Zachary F. Zimmerman [f], Keith Wilner [g], Willi Woessmann [h], Susan Weiner [i], Brenda Weigel [j], Rajkumar Venkatramani [k], Dominique Valteau [l], Toby Trahair [m], Malcolm Smith [n], Sonia Singh [o], Giovanni Selvaggi [p], Nicole Scobie [q], Gudrun Schleiermacher [r], Nicholas Richardson [o], Julie Park [s], Karsten Nysom [t], Koen Norga [u], Margret Merino [o], Joe McDonough [v], Yousif Matloub [w], Lynley V. Marshall [x], Eric Lowe [y], Giovanni Lesa [d], Meredith Irwin [z], Dominik Karres [d], Amar Gajjar [aa], François Doz [r], Elizabeth Fox [aa], Steven G. DuBois [ab], Martha Donoghue [o], Michela Casanova [ac], Hubert Caron [ad], Vickie Buenger [ae], Diana Bradford [o], Patricia Blanc [af], Amy Barone [o], Gregory Reaman [o], Gilles Vassal [a,l]

N°7 CART-cells

European Journal of Cancer 160 (2022) 112–133

Available online at www.sciencedirect.com

ELSEVIER

Review

Paediatric Strategy Forum for medicinal product development of chimeric antigen receptor T-cells in children and adolescents with cancer ACCELERATE in collaboration with the European Medicines Agency with participation of the Food and Drug Administration[2]

Andrew DJ. Pearson [a,*,1], Claudia Rossig [b,1], Crystal Mackall [c,1], Nirali N. Shah [d,1], Andre Baruchel [e,1], Gregory Reaman [f], Rosanna Ricafort [g], Delphine Heenen [h], Abraham Bassan [i], Michael Berntgen [j], Nick Bird [k], Eric Bleickardt [l], Najat Bouchkouj [f], Peter Bross [f], Carrie Brownstein [m], Sarah Beaussant Cohen [n], Teresa de Rojas [a], Lori Ehrlich [f], Elizabeth Fox [o], Stephen Gottschalk [o], Linda Hanssens [p], Douglas S. Hawkins [q], Ivan D. Horak [r], Danielle H. Taylor [s], Courtney Johnson [f], Dominik Karres [t], Franca Ligas [t], Donna Ludwinski [u], Maksim Mamonkin [v], Lynley Marshall [w], Behzad K. Masouleh [x], Yousif Matloub [y], Shannon Maude [z], Joe McDonough [aa], Veronique Minard-Colin [ab], Koen Norga [ac], Karsten Nysom [ad], Alberto Pappo [o], Laura Pearce [ae], Rob Pieters [af], Martin Pule [ag], Alfonso Quintás-Cardama [ah], Nick Richardson [f], Martina Schüßler-Lenz [ai,as], Nicole Scobie [aj], Martina A. Sersch [ak], Malcolm A. Smith [al], Jaroslav Sterba [am], Sarah K. Tasian [z], Brenda Weigel [an], Susan L. Weiner [ao], Christian Michel Zwaan [af,ap,ar], Giovanni Lesa [t], Gilles Vassal [a,aq]

Prioritising BET inhibitors

European Journal of Cancer 146 (2021) 115–124

Available online at www.sciencedirect.com

ScienceDirect

Review

Bromodomain and extra-terminal inhibitors—A consensus prioritisation after the Paediatric Strategy Forum for medicinal product development of epigenetic modifiers in children—ACCELERATE

Andrew DJ. Pearson [a,*], Steven G. DuBois [b], Vickie Buenger [c], Mark Kieran [d], Kimberly Stegmaier [b], Pratiti Bandopadhayay [b], Kelly Bennett [e], Franck Bourdeaut [f], Patrick A. Brown [g], Louis Chesler [h], Jessica Clymer [b], Elizabeth Fox [i], Christopher A. French [j], Eva Germovsek [k], Francis J. Giles [l], Julia G. Bender [m], Maureen M. Hattersley [n], Donna Ludwinski [o,p], Katarina Luptakova [q], John Maris [r], Joe McDonough [s], Zariana Nikolova [t], Malcolm Smith [u], Athanasios C. Tsiatis [v], Rajeev Vibhakar [w], Susan Weiner [x], Joanna S. Yi [y], Fred Zheng [z], Gilles Vassal [a,aa]

All available @

www.accelerate-paltform.org

ACCELERATE

INNOVATION FOR CHILDREN AND ADOLESCENTS WITH CANCER

c4c Multi-Stakeholder Meetings

MSM #1 - Paediatric Inflammatory bowel disease
14-15 April 2021, Virtual

MSM #2 - Pediatric Atopic Dermatitis
1-2 March 2022, Virtual

MSM#3 on medical devices in type I diabetes
21 February 2023, Berlin & Zoom

MSM #4 on Perinatal Asphyxia
18-19 Sep 2023, Rome & virtual

MSM #5 on Irritability
18-19 March 2024, Nice & virtual

674 PARTICIPANTS

366 ACADEMIA (54%)
130 INDUSTRY (19%)
75 REGULATORS (11%)
103 PATIENTS PARENTS (15%)

The C4C MSM Experts

Inflammatory Bowel Disease


Lissy de Ridder
Erasmus MC


Chrissi Pallidis
EMA


Marco Greco
European Patients' Forum


Nick Croft
Queen Mary UoL


Tara Altepeter
FDA


R. "Skip" Nelson
Johnson & Johnson


Wallace Crandall
Eli Lilly & Co.

Atopic Dermatitis


Suzanne Pasmans
Erasmus MC

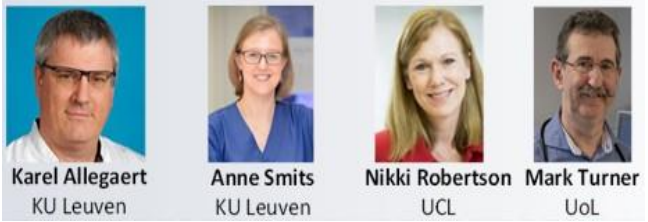

Sebastien Barbarot
CHU Nantes


Bernd Arents
VMCE


Dobromir Penkov
EMA


Janelle Burnham
Pfizer

Perinatal Asphyxia


Karel Allegaert
KU Leuven

Anne Smits
KU Leuven

Nikki Robertson
UCL

Mark Turner
UoL

aisling walsh
EFCNI

Ralph Bax
EMA

An Massaro
FDA

Type I Diabetes


Thomas Danne
HKA


Eric Renard
CHU Montpellier


Torben Biester
HKA


Felix Resche
HKA


Maren Sturny
Dedoc


Robert Geertsma
RIVM


Michelle Katz
Eli Lilly


Elizabeth Niemoeller
Sanofi


Marc Julien
Diabeloop

Irritability


Carmen Moreno
CUSM


Sara Carucci
UNICA


Benedetto Vitiello
UNITO


Florence Butlen
EMA

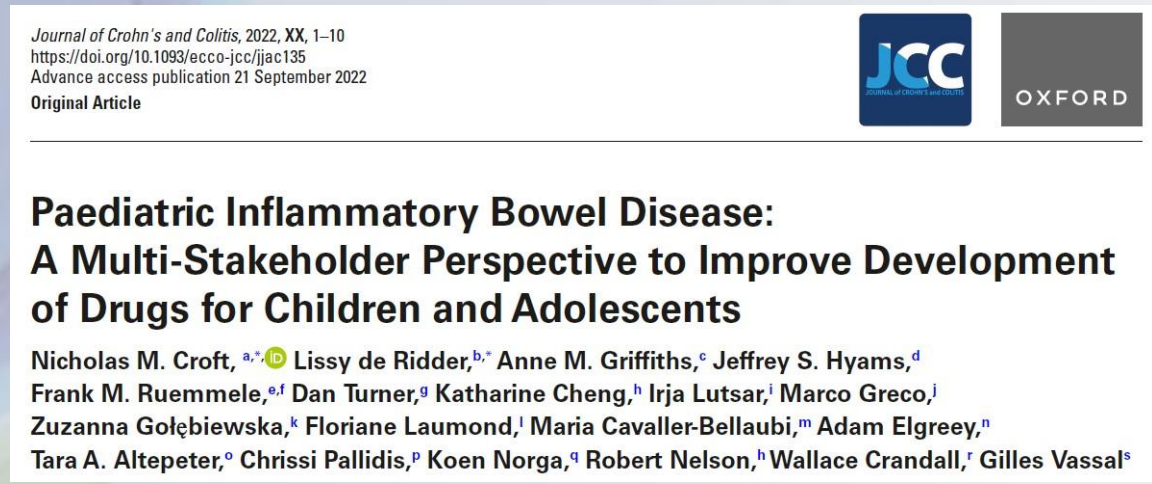

Argyris Stringaris
UCL


Christoph U. Correll
Charitè


Fernando Pontes Soares
Servier

c4c multistakeholder meetings publications

MSM #1



Journal of Crohn's and Colitis, 2022, **XX**, 1–10
https://doi.org/10.1093/ecco-jcc/jjac135
Advance access publication 21 September 2022
Original Article

Paediatric Inflammatory Bowel Disease: A Multi-Stakeholder Perspective to Improve Development of Drugs for Children and Adolescents

Nicholas M. Croft, [a,*] Lissy de Ridder,[b,*] Anne M. Griffiths,[c] Jeffrey S. Hyams,[d] Frank M. Ruemmele,[e,f] Dan Turner,[g] Katharine Cheng,[h] Irja Lutsar,[i] Marco Greco,[j] Zuzanna Gołębiewska,[k] Floriane Laumond,[l] Maria Cavaller-Bellaubi,[m] Adam Elgreey,[n] Tara A. Altepeter,[o] Chrissi Pallidis,[p] Koen Norga,[q] Robert Nelson,[h] Wallace Crandall,[r] Gilles Vassal[s]

MSM #2 - Pediatric Atopic Dermatitis – To be submmitted

MSM #3 on medical devices in type I diabetes – Submitted

MSM #4 on Perinatal Asphyxia – In writing

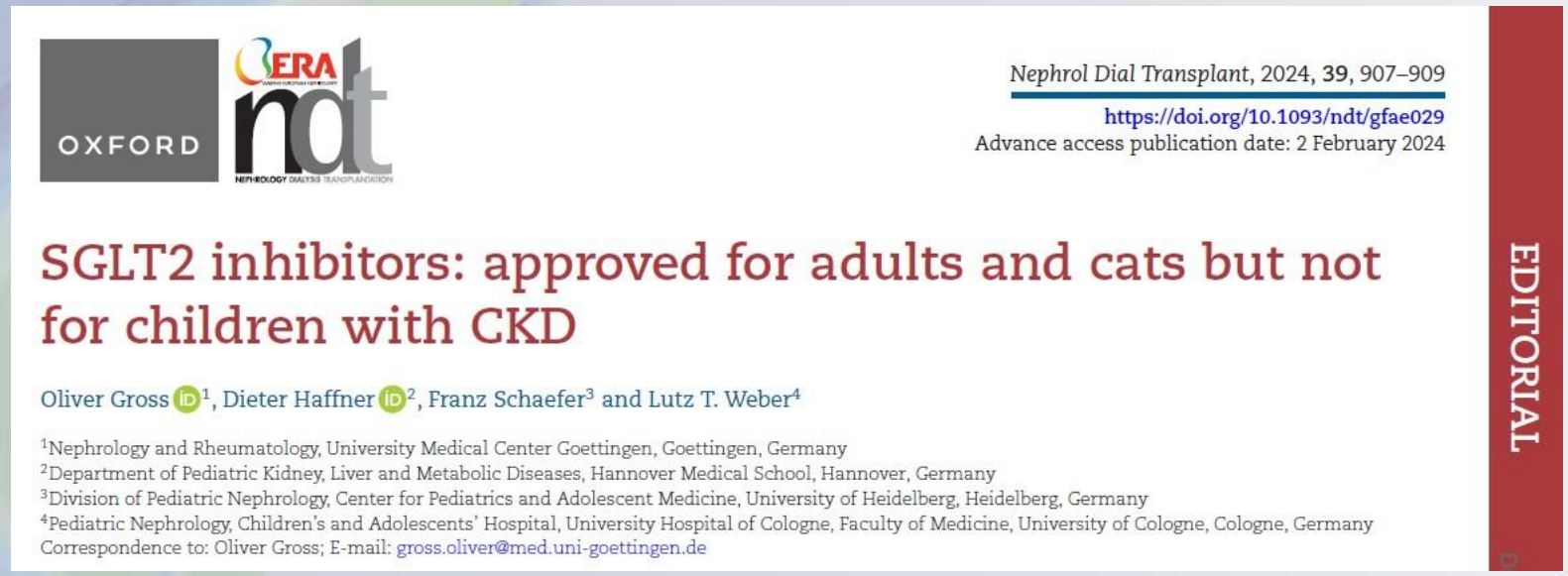
MSM #5 on Irritability – In writing

The 6th C4C multistakeholder meeting

Chronic Kidney Disease

April 2025, Heidelberg & virtual

Ahead of the ESCAPE meeting



OXFORD ERA ndt

Nephrol Dial Transplant, 2024, **39**, 907–909
https://doi.org/10.1093/ndt/gfae029
Advance access publication date: 2 February 2024

EDITORIAL

SGLT2 inhibitors: approved for adults and cats but not for children with CKD

Oliver Gross [1], Dieter Haffner [2], Franz Schaefer[3] and Lutz T. Weber[4]

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[2]Department of Pediatric Kidney, Liver and Metabolic Diseases, Hannover Medical School, Hannover, Germany
[3]Division of Pediatric Nephrology, Center for Pediatrics and Adolescent Medicine, University of Heidelberg, Heidelberg, Germany
[4]Pediatric Nephrology, Children's and Adolescents' Hospital, University Hospital of Cologne, Faculty of Medicine, University of Cologne, Cologne, Germany
Correspondence to: Oliver Gross; E-mail: gross.oliver@med.uni-goettingen.de

imi efpia conect 4children

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

6 February 2023
EMA/635567/2022
Paediatric Medicines Office

Boosting the development of medicines for children

Closing report of the European Medicines Agency and European Commission (DG Health and Food Safety) action plan on paediatrics

EUROPEAN MEDICINES AGENCY
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1. Identifying paediatric medical needs

	Action	Objectives	Achievements by January 2023
1	Develop overview of selected therapeutic areas to identify paediatric medical needs. Actions include: • Conducting public survey on criteria proposed for determining paediatric medical needs and on perceived areas of needs • Selecting therapeutic areas based on various factors, including experience with PIPs and stakeholder feedback, for further analyses by multi-stakeholder focus groups • Conducting multi-stakeholder workshops in selected therapeutic areas • Publishing reports on the paediatric therapeutic landscape related to selected areas	To raise awareness for paediatric medical needs with a view to providing a basis for strategic decision making on paediatric medicine development.	• Strategies to address needs in children with malignancies were determined at multi-stakeholder forums organised by EMA together with the ACCELERATE platform: – ACCELERATE & EMA Paediatric Strategy Forum for medicinal product development of checkpoint inhibitors for use in combination therapy in paediatric patients (09/2018), related publication in Eur J Cancer (11/2019). – ACCELERATE & EMA Paediatric Strategy Forum for medicinal product development for acute myeloid leukaemia in children and adolescents (04/2019), related publication in Eur J Cancer (09/2020). – ACCELERATE & EMA Paediatric Strategy Forum for epigenetic modifiers in paediatric malignancies (01/2020), related publication in Eur J Cancer (11/2020). – ACCELERATE & EMA Paediatric Strategy Forum on CAR T-cell developments (5/2021), related publication in Eur J Cancer (11/2021). ***new*** – ACCELERATE & EMA 2nd Paediatric Strategy Forum on ALK inhibitors (06/2021), related publication in Eur J Cancer (11/2021). ***new***
			focused workshops during 2020/21 in collaboration with the IMI c4c project. – Contribution to a multi-stakeholder workshop on paediatric inflammatory bowel disease in collaboration with the IMI c4c project (04/2021). ***New*** – Contribution to a follow-up multi-stakeholder workshop on paediatric unmet needs (06/2021). ***New*** – Contribution to a multi-stakeholder meeting on priorities for antiretroviral drug optimisation in children (Paediatric Antiretroviral Drug Optimization [PADO] meeting) (09/2021). ***New*** – Contribution to a multi-stakeholder workshop on paediatric atopic dermatitis in collaboration with the IMI c4c project (03/2022). ***new***

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Paediatric Strategy Forum:
Mature B cell malignancies in children
13 & 14 November 2017

Results and impact

Paediatric Strategy Forum: Mature B cell malignancies in children

13 & 14 November 2017

Participants

- 73 Participants
- European and North American academic experts
- Patient representatives from Unite2Cure (Europe) and Children's Cause for Cancer Advocacy (US)
- Regulators from EU national competent authorities, EMA & US FDA; and PDCO members
- 15 Pharmaceutical companies
- 20 Medicinal products discussed

- Highly effective current therapy for high-risk mature B cell malignancies with EFS~ 95% and no salvage therapy in relapse
- Acute toxicity – significant, but most survivors - no or mild long term toxicity

Unmet therapeutic needs

i) develop innovative treatments for patients remaining incurable

ii) reduce high acute toxicity of current therapy

Conclusion

- Most malignancies in children differ from those in adults
- Successful de-escalation at low risk in front line therapy can only be undertaken with an effective salvage regimen
- Priority = developing treatment for relapse
  - Very small number of patients = global strategy
  - Combination approach rather than monotherapy
  - Correlative biology sudies should be integrated

GLO-BNHL

Consensus of clinicians on priorities

- Antibody drug conjugates
- CAR-T cells (not products for initial use but only for consolidation)
- T-cell Engagers

PIP/waiver

Results

Anticancer drugs for non-Hodgkin lymphoma in adult

July 2007 –November 2017

- # of EMA decisions for Paediatric Investigation Plans ⇨ 15/27 (56%)
- # of EMA decision for Full-waivers ⇨ 12/27 (44%)

July 2015- July 2018 Revision of the class waiver list (CW/0001/2015)

November 2017- 2nd Paediatric Strategy Forum

December 2017 – June 2021

- # of EMA decisions for Paediatric Investigation Plans ⇨ 9/36 (25%)
- # of EMA decisions for Full-waivers ⇨ 27/36 (75%)

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INNOVATION FOR CHILDREN AND ADOLESCENTS WITH CANCER

OXFORD

JNCI: Journal of the National Cancer Institute, 2024, 116(2), 200–207
https://doi.org/10.1093/jnci/djad239
Advance Access Publication Date: November 17, 2023
Review

Impact of ACCELERATE Paediatric Strategy Forums: a review of the value of multi-stakeholder meetings in oncology drug development

ACCELERATE in collaboration with the European Medicines Agency with participation of the Food and Drug Administration

Andrew D.J. Pearson, MBBS, MD, DCH,[1,*] Teresa de Rojas, MD, PhD,[1] Dominik Karres, MD,[2] Gregory Reaman, MD,[3] Nicole Scobie,[4] Elizabeth Fox, MD,[5] Giovanni Lesa, PhD,[2] Franca Ligas, MD,[2] Koen Norga, MD, PhD,[6,7,8] Karsten Nysom, MD, PhD,[9] Alberto Pappo, MD,[5] Brenda Weigel, MD, MSc,[10] Susan L. Weiner, PhD,[11] Gilles Vassal, MD, PhD[1,12]

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Conclusions

- The EU regulatory landscape is changing
- Demonstrated feasibility and value of multistakeholder meetings to support science-based and patient-centric pediatric drug development and to facilitate prioritization
- In this changing regulatory environment
- MSMs are adapted to each topic/disease addressed

MSM and Rare Diseases

- What potential role to improve and accelerate therapeutic innovations for patients with rare and ultra rare diseases?
- What are the needs?
- How MSM could strengthen interactions between ERNs and Industry (and regulatory bodies) with a strong insight of patients and advocates?