The Pediatric Praziquantel Consortium
Helping children with Schistosomiasis

On behalf of the Consortium,
Dr. Remco de Vrueh (Lygature)
Consortium coordinator

December 8th, 2017
GHIT Fund R&D Forum, Tokyo
A new public-private partnership to address a high unmet medical need in young children

High Prevalence

High Medical need

Pre-school aged children neglected

Estimated 27 Mio young children infected or at risk of schistosomiasis in Sub-Saharan Africa

Current treatment (praziquantel 600mg tablets) cannot be administered to young children

→ clear unmet medical need for a suitable pediatric formulation for the children aged 3 months to 6 years
Founded in 2012

The Pediatric Praziquantel Consortium

- International non-profit R&D Consortium with a focus on extended partnership into endemic countries
- In kind and/or in cash contribution
- Continually seeking funding and advice from external experts and partners

International Expert Panel (World Health Organization) as observer

Grants:
- Bill & Melinda Gates Foundation
- GHIT Fund (3x)

Swiss TPH

Consortium Board

Consortium Team

KEMRI (Kenya)

Université Félix Houphouët-Boigny (Cote d’Ivoire)

Exit begin of Oct 2017

To join end of Dec 2017
True innovation

L-PZQ and rac-PZQ oro-dispersible tablets (ODTs)

- Orally disintegrating
- Improved taste
- Conventional manufacturing process

Cesol 600 mg
Current commercial tablet

PZQ new ODT
150 mg tablet
Full clinical development program for PZQ-ODTs

Completed (2015)
Two Phase I Bioavailability studies (South Africa)

Completed (2015)
Taste Study of the new ODTs in African children (Tanzania)

Ongoing (2016)
Phase II PK/PD dose finding Study (Ivory Coast)

Planned (2018)
Phase III confirmatory trials (Kenya/Ivory Coast)
Capacity building - Strengthening Health infrastructure

Clinical research facility annex refurbished (Ivory Coast - Man region)

BEFORE

11.03.2016, pre-study visit

AFTER

15.06.2016, initiation visit
Capacity building - Strengthening R&D infrastructure

Study team supervised by the PI (Prof. N’Goran), members of the Merck & Swiss TPH team

Blood sampling and Dry Blood Spot method
Next steps: Regulatory & Manufacturing plan

WHO EOI

Clinical program

1st registration EMA article 58 and WHO Pre-qualification

Registration in African endemic countries (WHO collaborative procedure)*

Launch in first African endemic countries

Safety database build up
Conduct Pharmacovigilance study with WHO involvement

Start Procurement for individual case management

Manufacturing launch readiness (Farmanguinhos)

Tech Transfer to local African manufacturer (tbd)

2016-2019
End 2019
End 2020
End 2020
2019
2020
2020
2023

Large scale distribution for prophylactic use

2016 - 2019
2019
2020
2023
One key for success

Communication & stakeholder engagement

Access expert meeting in Q3 2018

Key stakeholders: Consortium partners, our funders, NTD control program funders, WHO, UNICEF, KOLs and possibly endemic country decision makers