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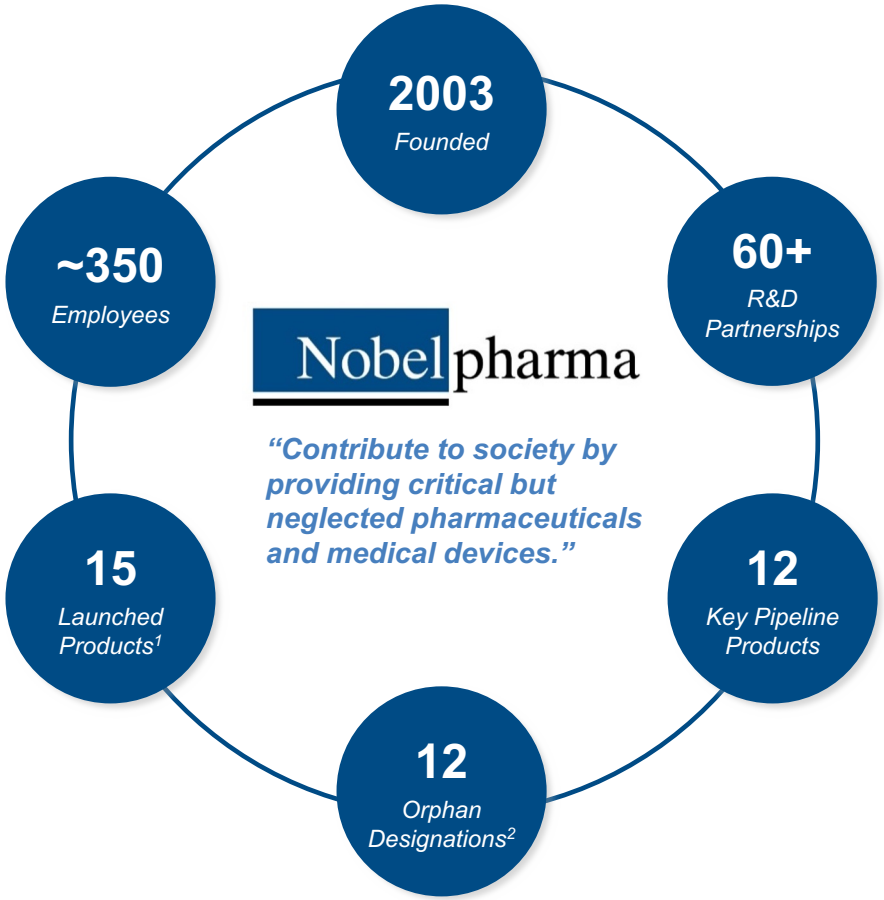
# Session 5: EVI and PATH MVI Developing Effective and Affordable Vaccines through Partnerships

# Our Company Mission

**Contribute to society by providing critical but neglected pharmaceuticals and medical devices**

# Nobelpharma has focused on the area of Rare and Orphan Diseases




**Proven Track Record**

- **Has launched drugs for rare diseases such as Wilson's disease, tuberous sclerosis-associated skin lesions, among others**

**Differentiated R&D Platform**

- **Development exclusively with external partners**
- **Specializing in Late-phase Development**

**Outsourcing Manufacturing**

- **Outsourcing manufacturing to external partners to pursue an asset light business model**

**Strong Distribution Capabilities**

- **Exclusive partnership with MEDIPAL HOLDINGS, one of Japanese leading pharmaceutical distributors**

Note: (1) Number of products launched by NPC
(2) Including products under development



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# When/How the collaborative work started with EVI

- In Feb 2012, Prof. Horii, Osaka University, and Dr. Leroy, EVI, met first time during WHO Malvac meeting in Geneva
- In Aug 2013, an introductory letter was sent to Prof. Horii from Dr. Stefan Jungbluth introducing EVI as a Product Development Partnership.
- In Sept 2013, first T-con to discuss potential collaboration
- EVI introduced Dr. Sodiomon Sirima, CNRFP (now Chief Executive Officer @Gras) in Burkina Faso, to Prof. Horii – and the three entities agreed to come together with shared interest and strong commitment to develop NPC-SE36 vaccine candidate
- In Apr 2014, the three partners applied for GHIT Fund. The proposal was approved in Aug 2014 and the grant agreement was signed. The CONSORTIUM agreement between partners was signed in August 2014 and a clinical trial agreement was in place in November 2014.
- Just at that time, Nobelpharma joined this CONSORTIUM



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# Burkina Faso

- Name was changed from “Upper Volta” by President Thomas Sankara
- Population : 22.7M (2019)
- Capital : Ouagadougou

Clinical trials in Burkina Faso
- ☆ P1b/AHG in Banfora
- ☆ P1b/CpG in Ouagadougou


AFRICA




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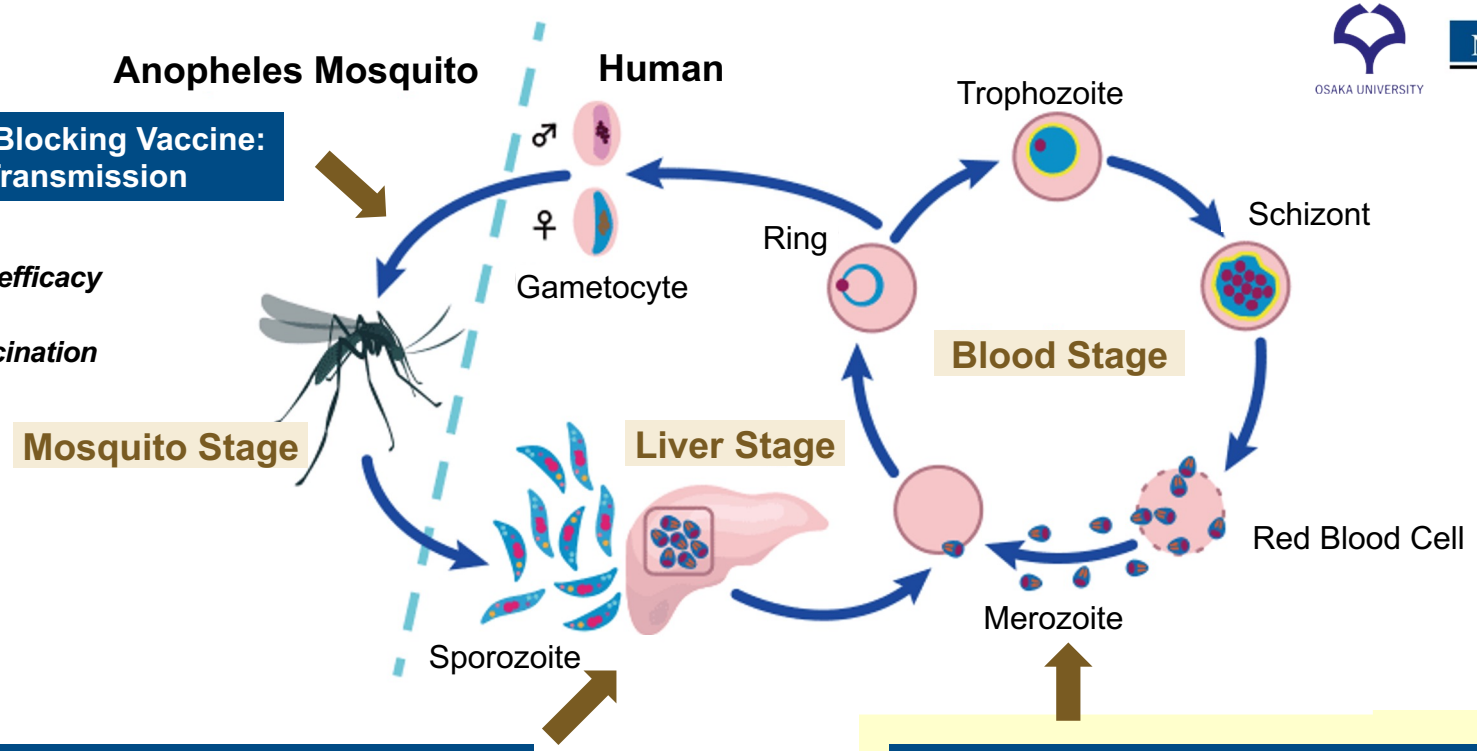
# Malaria Parasites Life Cycle and Potential Vaccine Targets




**Transmission Blocking Vaccine: Prevent Transmission**

***Challenge:***

- ***Difficulty in efficacy evaluation***
- ***"Mass" vaccination***

**Liver Stage Vaccine: Prevent Infection**

**RTS,S/AS01**

**R21/Matrix-M**

***Limitation:***

- ***High polymorphism of csp***
- ***Escape parasite can lead to blood stage infection***

**Blood Stage Vaccine: Prevent Disease**

**NPC-SE36**

***Advantage:***

- ***Limited polymorphism of SE36***

***Challenge:***

- ***Low seroconversion in malaria highly exposed population***

Note: Revised scheme from original scheme in RIMD/Osaka University HP.

# CONSORTIUM organization, R&R

| | Designated Development Partner | Collaboration Partner 1 | Collaboration Partner 2 | Collaboration Partner 3 |
|---|---|---|---|---|
| Organization Name | European Vaccine Initiative (EVI) | Osaka University<br>• Research Institute for Microbial Diseases (RIMD)<br>• Medical Center for Translational Research (MTR) | Groupe de Recherche Action en Santé （GRAS) | Nobelpharma (NPC) |
| R&R | ✓ Overall coordination and management of the programme<br>✓ Representative funding recipient | ✓ Scientific developer<br>✓ Coordinate for immunogenicity assessments | ✓ Involved in Africa P2 protocol and study dossier preparation<br>✓ Help in trial site selection/evaluation process in Africa | ✓ Development partner and Sponsor<br>✓ Lead and provide GMP grade batch and CpG-ODN(K3) |



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# Contract Structure with GHIT Fund

## Investment Agreement

- ✓ Award of Budget to the CONSORTIUM
- ✓ Nomination of Partnership
- ✓ Requires Collaboration Agreement among the Partners
- ✓ Collaboration Partners Indemnification
- ✓ Payment and bi-Annual Progress Report schedules
- ✓ GHIT-monitored Milestones and Activities
- ✓ Requires Record Maintenance and Inspection by GHIT on how the Investment was expended
- ✓ Audit Rights to confirm the Partners compliance with Investment Agreement

## Collaboration Agreement

## Memorandum of Understanding

- ➢ among EVI, RIMD/Osaka Univ. and NPC in July 2018



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# EVI is responsible for and dedicate to:

★ Project governance (among others)
- ✓ troubleshoot partnership
- ✓ recommendation of expert pool (ISAC, DSMB, etc)
- ✓ engagement with GHIT, contractors and regulatory bodies if needed

★ Financial management
- ✓ as lead coordinator, receive and allocate the budget from GHIT to each Partner
- ✓ budget reporting

★ Communication
- ✓ reporting to GHIT
- ✓ annual and regular meetings with consortium partners

# NPC-SE36 Clinical Development History




**SE36/AHG**

**BK-SE36** preclinical studies

First clinical trials in humans **Phase 1a Japan**

**Phase 1b Uganda:** 6-40 y-old

Phase 1b **Follow-up study** 6-20 y-old

**Phase 1b Burkina Faso:** 1-5 years-old

**2003** **2005** **2007** **2010** **2011** **2013** **2015** **2018** **2020**

Early development


**SE36/CpG**

**Phase 1b Burkina Faso:** 1-45 y-old

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# NPC-SE36 Phase 1b study Outline

| | | |
|---|---|---|
| Indication | : | Malaria vaccine (*P. falciparum* clinical malaria) |
| Stage | : | Phase 1b |
| Study Period | : | May 2018 – April 2020 |
| Study Site | : | One site in Burkina Faso |
| Study Population | : | Cohort 1 : 21 - 45 years, n=45 (A:C=2:1) |
| | | Cohort 2 : 5 - 10 years of children, n=45 (A:C=2:1) |
| | : | Cohort 3 : 12 - 24 months of children, n=45 (A:C=2:1) |
| Vaccination schedule | : | Three immunizations on 0W, 4W and 16W |
| Observation period | : | 12 month after 1st immunization |
| Primary objectives | : | Safety, assess solicited and unsolicited AEs and SAEs |
| Secondary objectives | : | Anti-SE36 protein IgG antibody titres |
| Exploratory objectives | : | Preliminary vaccine efficacy against naturally occurring *P. falciparum* infection |