



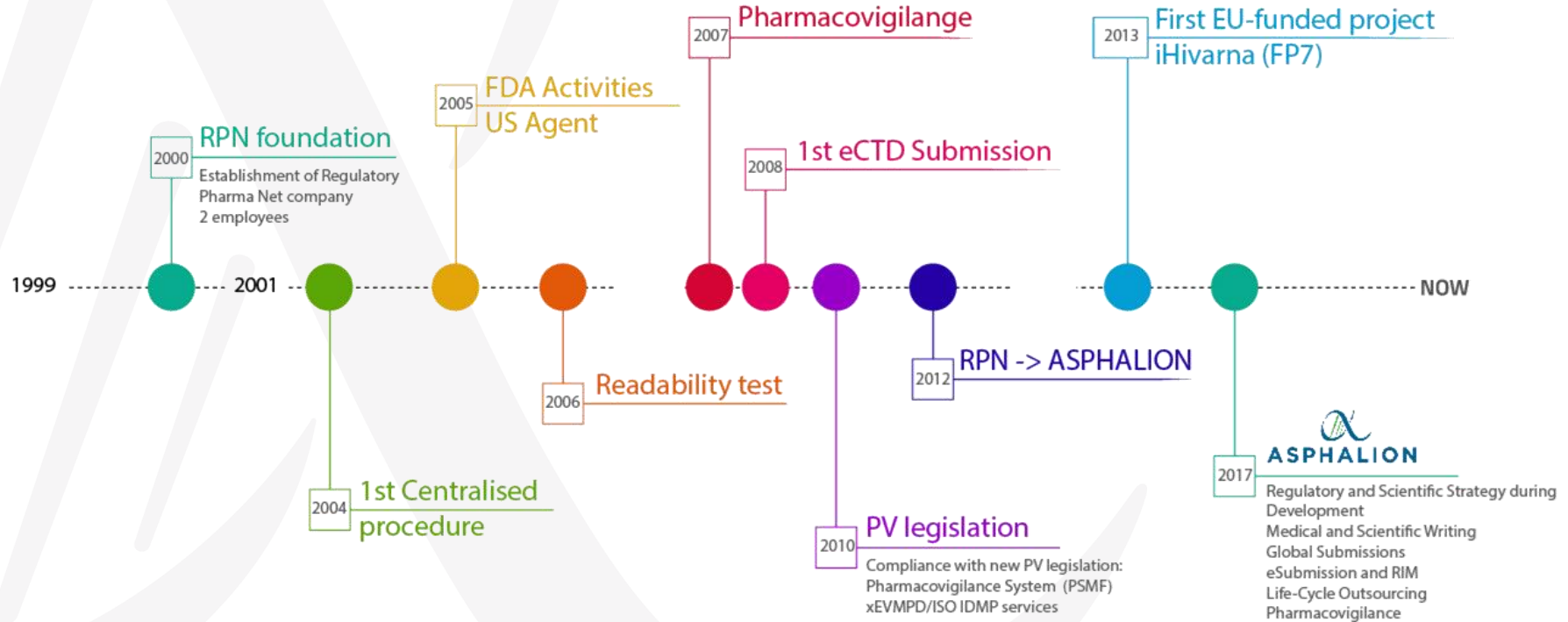
ASPHALION

**Horizon 2020 Health
Summit-IIS La Fe**

**Valencia, 12 de diciembre de
2017**



History



**+75
members**

**Consultants
with Life
Science
Background**

**20 team
members
with PhD**

**High
knowhow in
drug
development
and R&D**

**+25 years
accumulated
expertise**



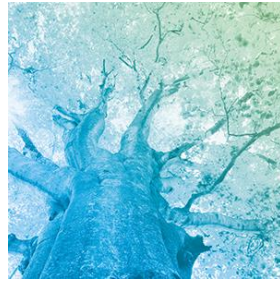
Asphalion services



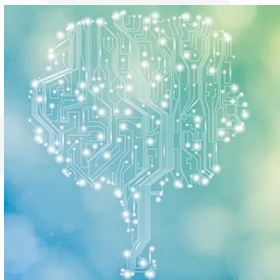
Regulatory & Scientific
Strategy



Medical and
Scientific Writing



Global
Submissions



eSubmission and RIM



Life-Cycle

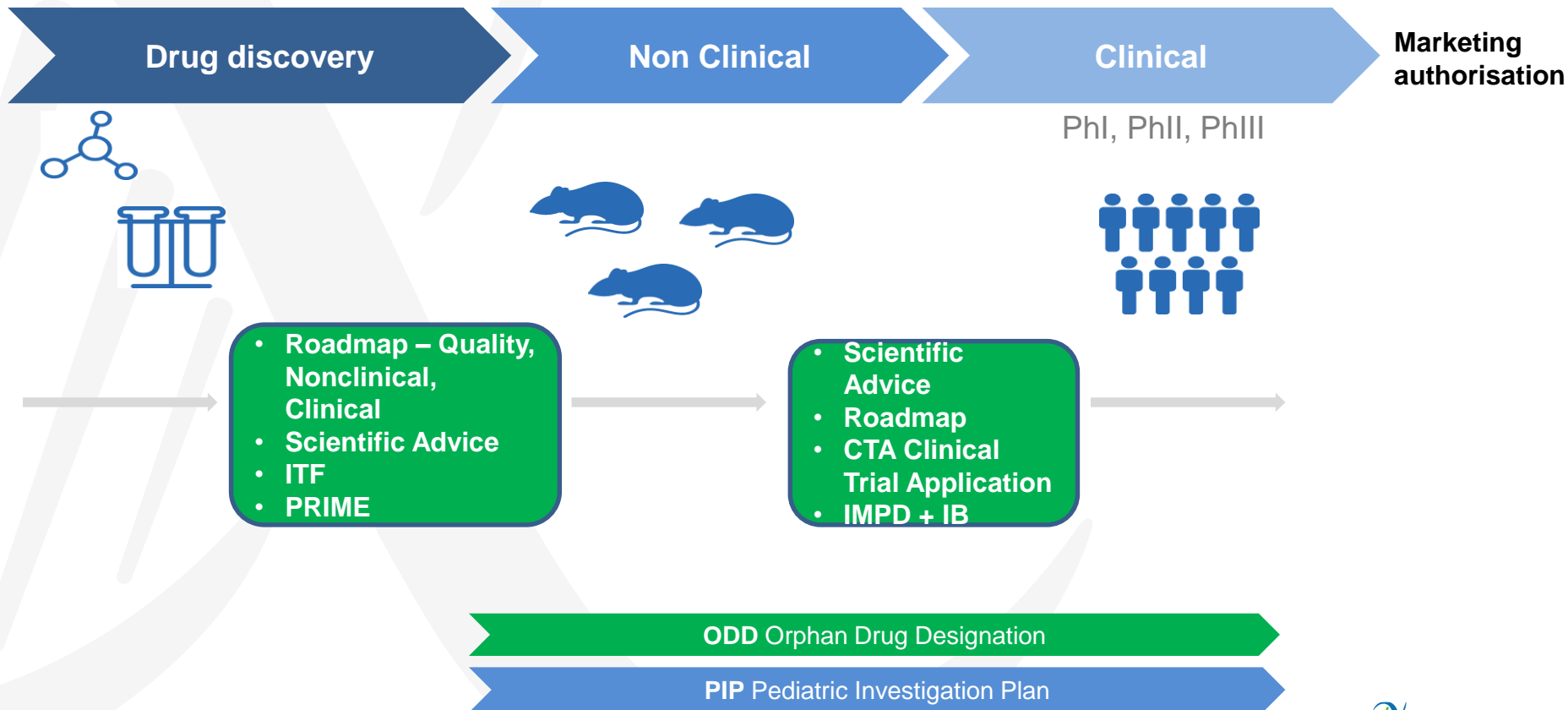


Pharmacovigilance

Drugs
Biologics
ATMPs
Medical Devices

Asphalion expertise in early drug development and H2020 projects

Regulatory Support during Development



Regulatory and Scientific Strategy during Development



Establish the right strategy by relying on experts

Roadmaps: non-clinical and clinical development plans, including assessment of feasibility, timelines and costs

Scientific Advice: expertise for optimal outcome with EMA, EU-NCAs, FDA and other agencies

Orphan Drug Designation application and **Paediatric Investigation Plans (PIPs)**

Clinical trial documentation: **IMPD**, **IB**, **CTA** and **IND**

All **EU** and **FDA** procedures: minimum time-to-market and maximum cost efficiency

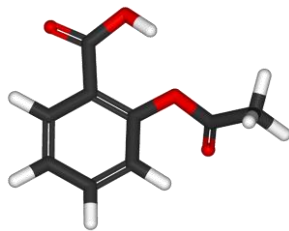
- ✓ **35 regulatory roadmaps** for early stage projects
- ✓ **More than 30 Scientific Advice**
- ✓ **Assistance in the preparation of 40 IMPD**
- ✓ **20 Orphan Drug Designations (EU/FDA)**

Product and therapeutic area expertise

Therapeutic areas

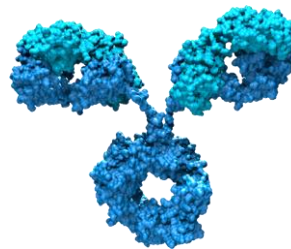
- Oncology
- HIV
- Dermatology
- Gastroenterology
- Analgesia
- Anti infective
- Respiratory
- Neurological disorders
- Metabolism
- Therapeutic vaccines
- Diabetes

NCE

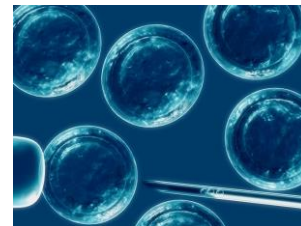


Innovative products

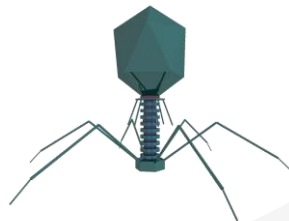
Biologicals



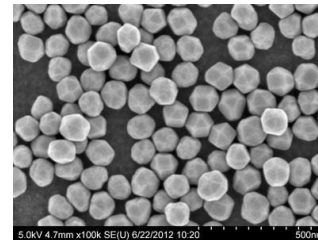
ATMPs



Vaccines



Nanoparticles and biomaterials



What we really do: how do we interact with investigators?

1. **Road maps:** Strategic regulatory and scientific assessment of a research project, from bench to bedside
2. **Medical writing:** document preparation and analysis
3. **Project management:** day to day input in running and moving projects forward
4. **Problem solving:** ad hoc consulting and troubleshooting for specific issues
5. **Research:** partnering in research consortium as regulatory experts

Asphalion expertise in H2020 projects

Asphalion participation in European projects

ASPHALION supports
research consortia as
SME Regulatory Expert Partner



- Experience in proposal preparation and application process since 2012
- First successful grant agreement in 2013 (iHIVARNA)
- Experience in 5 FP7/H2020 projects as regulatory partners
- Overall potential funds of over 24 million euros

Asphalion participation in European projects

Support during H2020 proposal writing

- Advise on the alignment of project idea with call text.
- Provision of proposal templates.
- Support in formatting and editing the proposal.
- Regulatory Work Package (WP) design and preparation. Budget estimation.
- Compilation and integration of WPs in the final proposal.
- Support to the coordinator in the proposal writing process.
- Critical revision of the final proposal.

Achievements

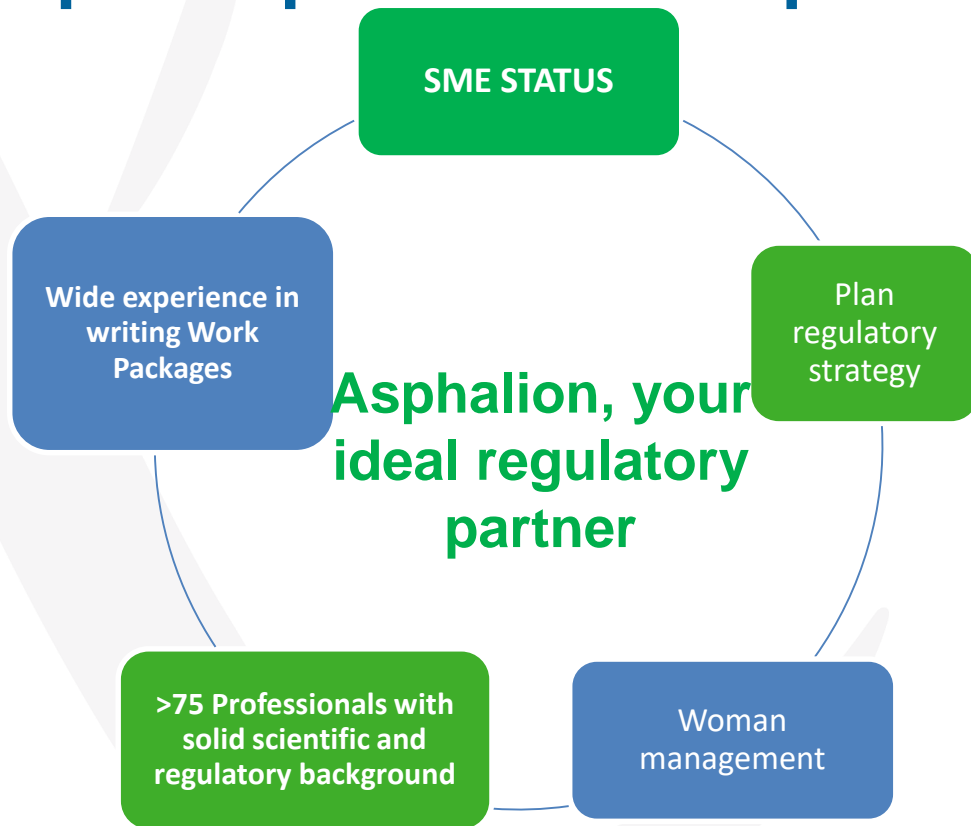
**4 H2020 funded projects
(SC1 and NMBP)**

1 FP7 funded project

30% success rate



Asphalion participation in European projects



On-going EU-funded projects



2013

Therapeutic TriMlx / mRNA based Vaccine in Chronic HIV-1 Infected Patients on Antiretroviral Therapy



2015

Development of an innovative gene therapy platform to cure rare hereditary muscle disorders



2015

An encapsulating strategy based on a bio-inspired material that mimics elastin



2017

Evaluating a Combination of Immune-based Therapies to Achieve a Functional Cure of HIV Infection



2017

Multimodal nanoparticles for structural and functional tracking of stem cell therapy on muscle regeneration

H2020 projects

Active in
2013



“Therapeutic TriMix/mRNA based Vaccine in Chronic HIV-1 Infected Patients on Antiretroviral Therapy”

The primary aim of iHIVARNA is to successfully immunize antiretroviral-treated HIV-infected patients with 3 injections of the candidate universal HIV-TriMix-mRNA as an mRNA-based therapeutic vaccine with the final aim of improving the efficacy of therapeutic vaccination against HIV infection.

Asphalion project contribution:

- 2 Scientific Advice at AEMPS (for Phase I and Phase II studies)
- GLP Toxicity design and follow-up
- 2 IMPD and 2 IB preparation
- Data analysis of the Phase I and Phase II clinical trial
- Ad-hoc regulatory advice

The research leading to this results has received funding from the European Community's Seventh Framework Programme (FP7/2007-2013) under grant agreement n° 602570 - the iHIVARNA project.

H2020 projects

Active in
2015



elastislet

“Tailored elastin-like recombinamers as advanced systems for cell therapies in diabetes mellitus”

The challenge consists in designing a capsule that ensures the appropriate interaction between transplanted pancreatic cells and the host tissues.

Asphalion project contribution:

- Regulatory Roadmap
- Report on CMC and non-clinical aspects with regulatory recommendations
- ITF consultation
- Request form for the ATMP classification
- Ad-hoc regulatory advice
- *Scientific Advice – to be performed*
- *IMPD preparation - to be performed*

This research project has received funding from the EU's H2020 framework programme for research and innovation under grant agreement n. 646075.

H2020 projects

*Active in
2015*



“Development of an innovative gene therapy platform to cure rare hereditary muscle disorders”

The main objective of MYOCURE is to develop a novel, innovative and clinically-translatable one-treatment gene therapy platform for patients suffering from rare inherited muscle diseases, specifically focusing on myotubular myopathy (MTM) and glycogen storage disorder (GSD) type II.

Asphalion project contribution:

- 2 Scientific Advice with the EMA
- Ad-hoc regulatory advice
- 2 Orphan Drug Designation Application - *to be performed*

This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 667751.

H2020 projects

Active in
2017

HIVACAR

Development of a Combination of Therapeutic Vaccine, Broadly Neutralizing Antibodies and Viral Reservoir Activators to Modify Latent Reservoirs in Chronic HIV-1 Infected

The main goal of HIVACAR is to change the current paradigm of HIV treatment by obtaining a functional cure for HIV (i.e., control of viral load to levels below the threshold of 50 copies/ml and maintenance of high CD4+ T-cell count after discontinuation of antiretroviral therapy) by effectively targeting residual virus replication and viral reservoirs.

Asphalion project contribution:

- ITF meeting
- Scientific Advice in Belgium
- Ad-hoc regulatory advice
- IMPD and IB preparation – *to be performed*

This Project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 731626.

H2020 projects

*Active in
2017*



Multimodal nanoparticles for structural and functional tracking of stem cell therapy on muscle regeneration

The main goal of nTRACK is to develop a safe and highly sensitive multimodal nanoimaging agent enabling noninvasive, quantitative and longitudinal stem cell tracking and whole body biodistribution. nTRACK will also provide information on cell (long-term) viability using the combination of CT, MRI and PET, which are imaging modalities that are clinically available.

Asphalion project contribution (recently granted)

- *Regulatory Roadmap including EMA and FDA regulatory frameworks.*
- *Scientific Advice procedure with the EMA*
- *Qualification of Novel methodologies*
- *IMPD preparation*

This project has received funding from the European Union's Horizon research and innovation programme under grant agreement No 761031.

2018-2020 Main topics for research institutes

Personalized medicine

2018

Human microbiome

2019

Combinatorial therapies

Co- and multimorbidities

Innovative health and care industry

2018

Advanced therapies

Regulatory Sciences

2019

Regenerative medicine

Next generation sequencing

Infectious diseases and improving global health

2018

Neglected infectious diseases

2019

HIV, tuberculosis (TB) and/or hepatitis C (HCV)

Antimicrobial drugs and vaccines development

*“Stimulating innovation in the European healthcare domain and industry by exploring the application of advanced technologies, improve the health of the workforce and promote **regulatory science**”*

Thank you!

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