asebio farmaindustria federación española de empresas de fenin TECNOLOGÍA SANITARIA MEDICAMENTOS INNOVADORES Plataforma Tecnológica Española

SOM BIOTECH

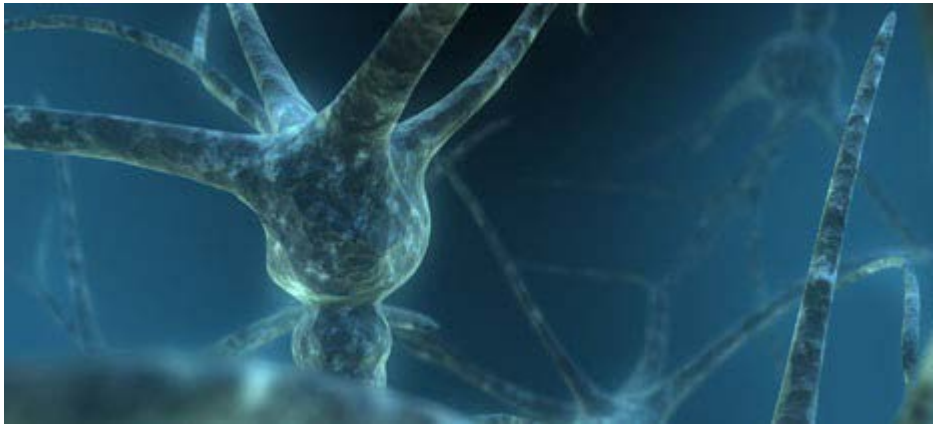
The drug repositioning company

VIII Conferencia Anual de Plataformas Tecnológicas de Investigación Médica

SESION 1: Los Pacientes en Investigación Clínica

El paciente de IC en la pequeña "biotech"

Dr. Raúl Insa, Fundador y CEO, insa@sombiotech.com 690 623 263



Plataforma de Mercados Biotecnológicos (Spanish Biotech Platform) NANOMED SPAIN PLATAFORMA ESPAÑOLA INNOVACIÓN TECNOLOGÍA SANITARIA IMIM Parc de Salut MAR Institut Hospital del Mar d'Investigacions Mèdiques GOBIERNO DE ESPAÑA MINISTERIO DE ECONOMÍA Y COMPETITIVIDAD

How small we are

MANAGEMENT TEAM

Raúl Insa, CEO, Founder
- MD in Clinical Neurology, ESADE, IESE, Harvard.
- 21 years: Parke-Da
- vis, UCB, Uriach, ISDIN.

Núria Reig, R&D Manager
- PhD in Biochemistry.
- 7 years: USA and Switzerland (Biotech).

Oscar Huertas, Senior Scientist
- BSc Computational Chemistry.
- 4 years experience: Intelligent Pharma.

Santiago Esteva, Business Development
- PhD in Biology. Master Pharma MRKT.
- 5 years experience in clinical CRO.

Richard Le, Research Fellow
- BSc and Master in Applied Science

David Gonzalvo, CFO
- Chartered Financial Analist, ESADE

Open position, Senior Scientist

STRATEGIC ADVISORY BOARD

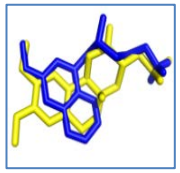
Joaquim Trias, PhD
- Bio entrepreneur
- San Francisco, US

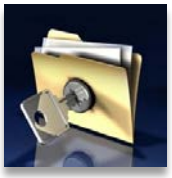
Catherine Miner, BSc, MBA
- Entrepreneur, Managing Partner WTCP
- Toronto, Canada.

Raj Airey, BSc, MBA
- Ex CEO in Pfizer, Baxter, others
- 26 years experience in license, M&A

Hermann A.M. Mucke , PhD
- Ex R&D Vice-president at Roche
- University of Vienna. Austria

Business Model; Drug Discovery to Clinical

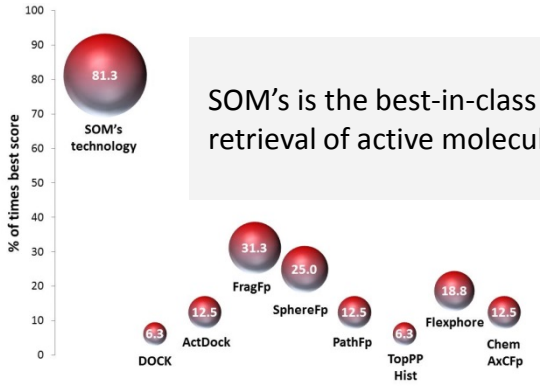




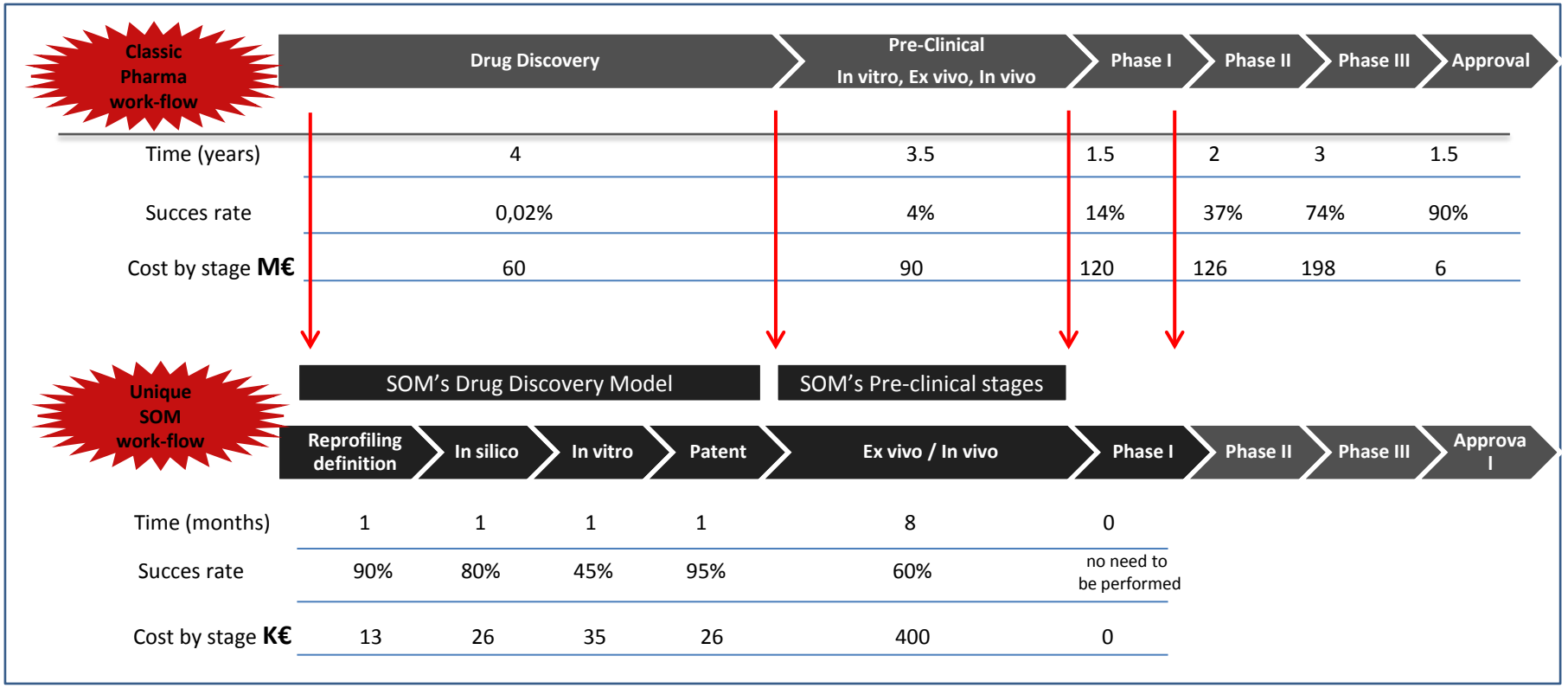

Commercial Drug Screening	Experimental Validation	IP Protection	License Phase-II POC
Ligand based *in silico* screening	Relevant *invitro, invivo* or *exvivo* models of the disease	WW mode of use and/or composition of matter patent	✓ Early licensing and add-hoc projects ✓ Late licensing

In House - In Property

- Ligand-based Virtual Screening Technology
- Molecular field maps through 22 molecular potentials
- 3D maps; flexible alignment; intensive in CPUs use
- Identification of non-structural analogs



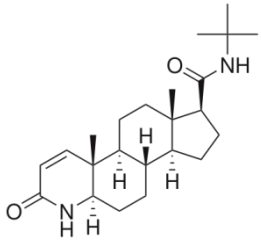
SOM's is the best-in-class in the retrieval of active molecules.



SOM's model vs. Classic Pharma model:

	SOM's model	Classic Pharma	Difference
Time to Phase II:	1 year	9 years	**8 years less**
Success to Phase II:	1/6	1/10.000	**1,666 times more**
Investment to Phase II:	€0.5M	€270M	**540 times less**

Success examples in repurposing



FINASTERIDE

Merck

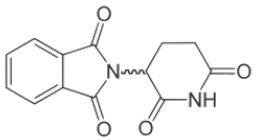
1992: Prostatic hypertrophy

17 €

REPROFILED

1998: Hair loss

52 €



TALIDOMID

Grünenthal

1957: Nausea

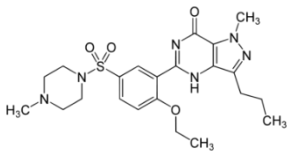
40 Pts

Celgene

2008: Leprosy / Oncology

Thalomid®

6.000 €



SILDENAFIL

Pfizer

1992: Hypertension

REPROFILED

1998: Erectile Dysfunction

99 €

REPROFILED

2012: Pulmonary Hypertension

24 €

Pipeline *December 2014*

AREA	PRODUCT	INDICATION	DISCOVERY	In Vitro VALIDATION	In Vivo VALIDATION	PHASE I*	PHASE II	STATUS
METABOLIC ORPHAN	SOM0226	*TTR Amyloidosis*						Phase IIa ongoing
NEUROLOGY ORPHAN	SOM3355	*Huntington*						Phase IIa ongoing
UROLOGY	Alpha-1A adrenergic antagonist	*B. Prostatic Hyperplasia*						Ex vivo studies ongoing
NEUROLOGY	Sigma-1 agonist	*Amnesia Alzheimer*						In vitro studies ongoing Novel MoA
ONCOLOGY ORPHAN	SOM0777	*Glioblastoma*				ARGON PHARMA		Licensed back

* As repositioned compounds, Phase I can be skipped

SOM0226: development plan

In-silico
Top 100 list
Report: MAR 2011

In vitro
Kinetic turbidity assay
Report: AUG 2011

IP
PCT Patent
Filed: OCT 2011

Ex-vivo
Steps of ATTR process:
T_4 competition, TTR stabilization, aggregation & fibril disruption
Active in FAC and FAP Mutations
Dose finding study in transgenic mice
Reports: 2012-2013

SOM0226-TTR Amyloidosis	2013	2014				2015				2016	2017	2018
	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Yr	Yr	Yr
Orphan Drug Status US	◆											
Orphan Drug Status EU												
EMA/FDA Advise							◆					
Biomarker validation												
Preclinical package												
Phase IIa POC EU V30M (n=20)												
OD formulation												
CMC development + Bioequivalence												
Clinical samples (for Phase IIb/III)												
IND/IMPD							◆					
Phase III Pivotal Studies												
NDA												
Approval												◆

SOM3355: development program

In silico

Top 100 list

2012

In vitro

VMAT2 inhibition assay

2013

IP

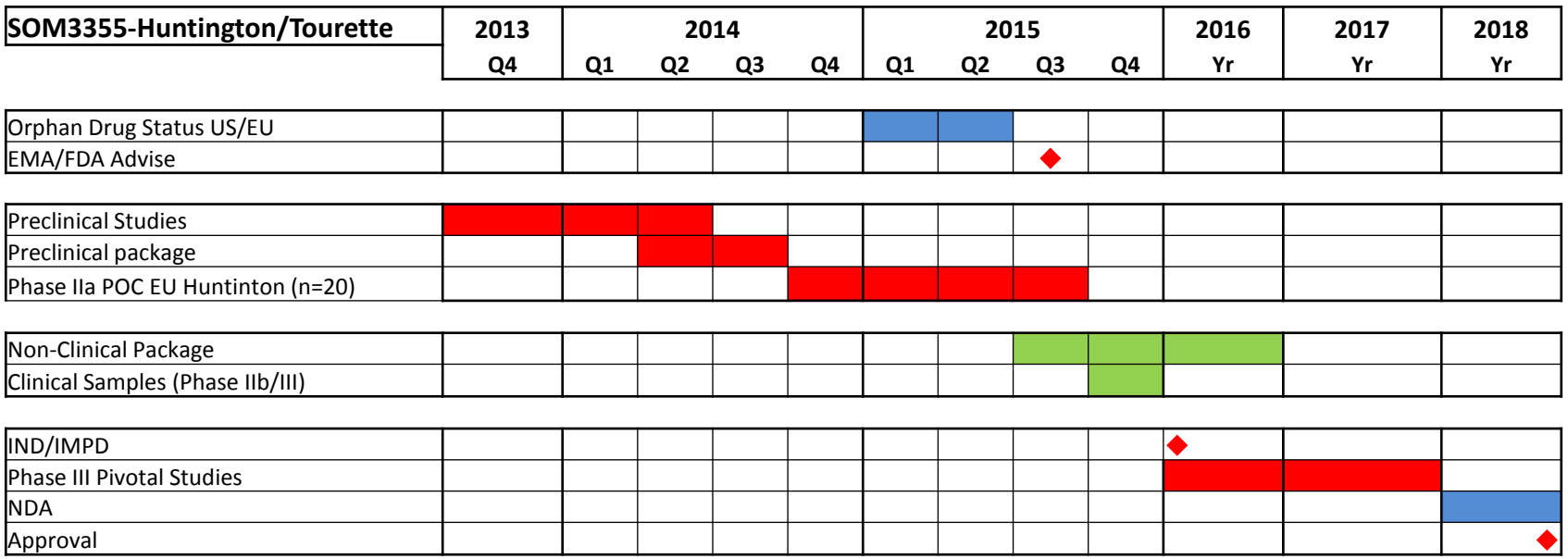
EP patent filed
'Therapeutic agents for use in the prophylaxis and/or treatment of hyperkinetic movement disorders'

JUN 2013

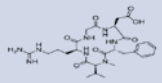
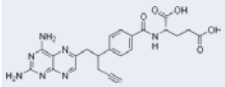
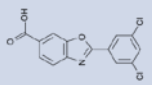
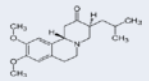
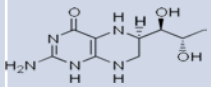
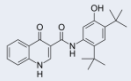
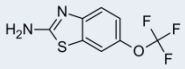
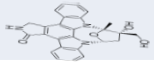
In vivo

- Quantification of SOM3355 brain levels after oral administration

2013-2014



Orphan pipeline

INDICATION	LIGANT	STRUCTURE	RESULTS	STATUS
Glioma (I)	Cilengitide		**SOM0777** Activity +++ Celular line +++	Non-exclusively licensed to Argon Pharma SL
T Cell Lymphoma	Pralatrexate		Three +++ hits identified	Abandoned. Already patented
TTR Amyloidosis	Tafamidis		**SOM0226** In vitro +++, Ex-vivo +++ Primary indication 1997	Open for out-license WW Patent ODS + Phase II POC ongoing
Huntington Disease	Tetrabenazine		**SOM3355** In vitro +++, BBB +++ Primary indication 1995	Open for license WW Patent Phase II POC under discussion
Phenylketonuria	Sapropterin		40 compounds under screening	No result yet
Cystic Fibrosis	Ivacaftor		Not yet	*In silico* process
Spinobulbar M Atrophy	*Undisclosure*	*Undisclosure*	Not yet	*In silico* process
Amyotrophic Lateral Sclerosis	Riluzole		Not yet	*In silico* process
Glioma (II)	*Undisclosure*	*Undisclosure*	Not yet	*In silico* process
Acute Myeloid Leukemia	Lestaurtinib		Not yet	*In vitro* process

Involvement of patients in CT (Orphan)

The ADVANTAGES to involve patients as soon as possible should be:

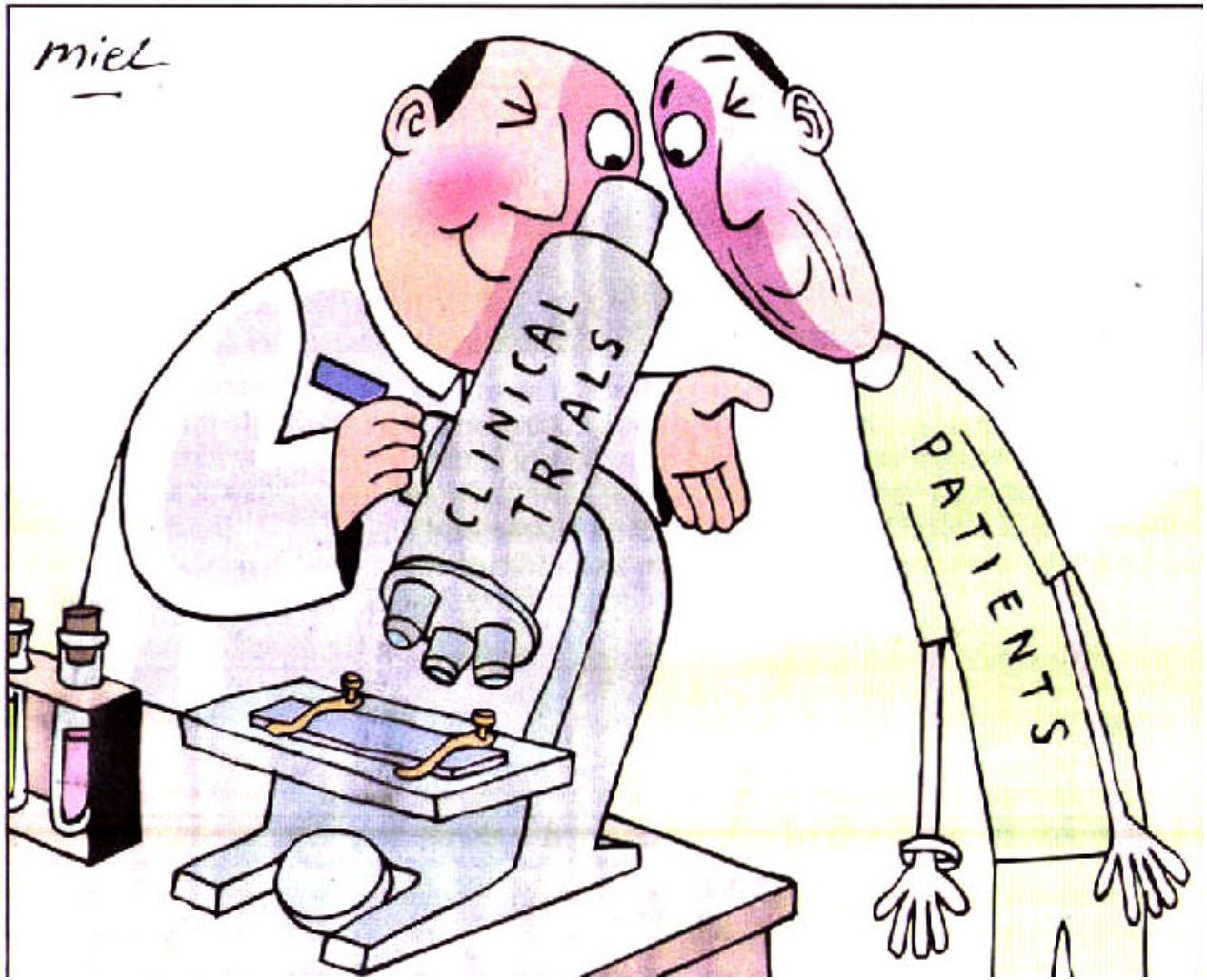
- ✓ **Participate in the Program Development** →
- ✓ **Participate in the Protocol Design** ↗
- ✓ **Roll in the Clinical Trial** ↑
- ✓ **Compassionate Use** ↑
- ✓ **MA Approval Process** →
- ✓ **Others: training, health-economics, academia…**

TIME & QUALITY

The CHALLENGES is whether the systems will:

- ➢ **Help small companies to identify patients**
- ➢ **Help patients to identify in which studies they can help**
- ➢ **Simplify patients access to centres in different regions**
- ➢ **Teach and form patients for a better understanding of the process**
- ➢ **Become a priority for the Health System**

Thanks for your time !



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