SOM3355, a promising repurposed candidate intended for the treatment of Huntington's disease and other related hyperkinetic movement disorders

Barcelona, 20 de octubre de 2015

Content

1. The Institution
2. The Product
   - Target Indications
   - Innovative mechanisms of action
   - Differential features facing the market
   - Current status of development
   - IPR protection
   - Pitfalls & Risks to be considered
3. Partnering Opportunities

1. The Institution

MANAGEMENT TEAM

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

David Gonzalvo, CFO
- Chartered Financial Analyst.
- ESADE Business & Law.

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.

Richard Le, Junior Scientist
- BSc and Master in Applied Science .

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

STRATEGIC ADVISORY

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

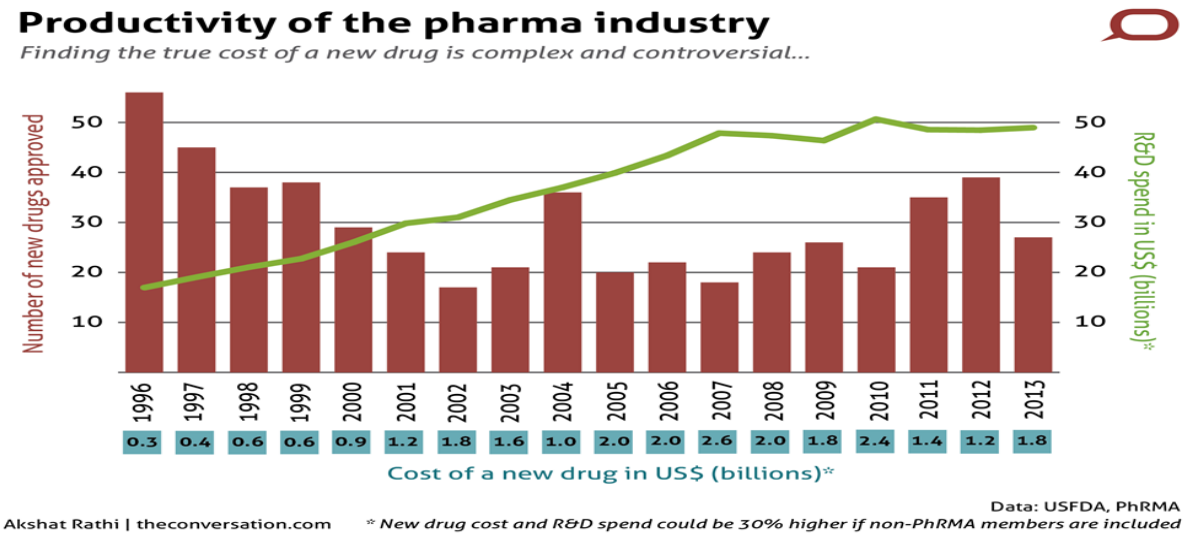
Catherine Miner, BSc, MBA
• Entrepreneur, Managing Partner WTCP
• Toronto, Canada.

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

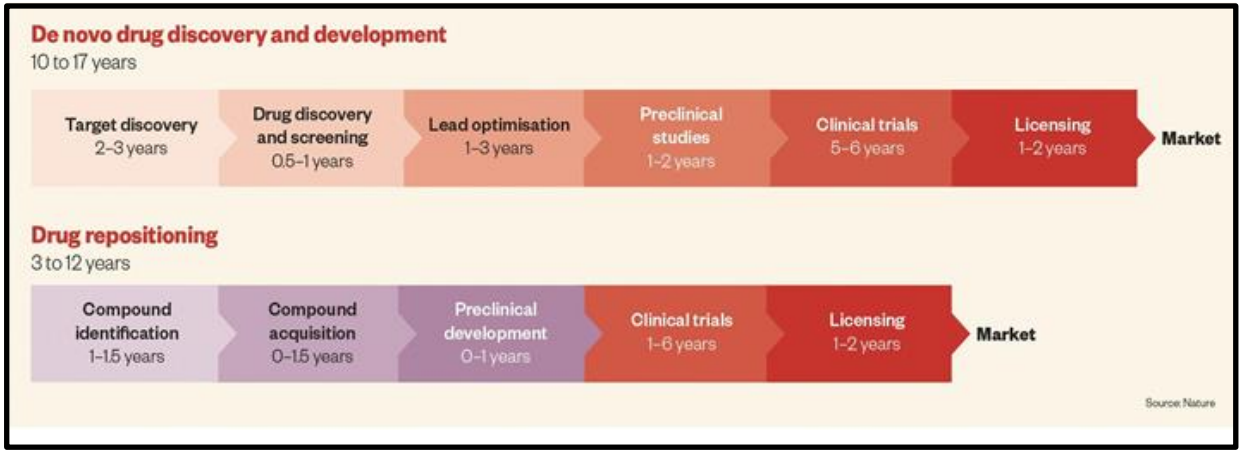
Hermann A.M. Mucke , PhD
• Ex R&D Vice-president at Roche
• University of Vienna. Austria

KEY SHAREHOLDERS

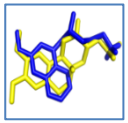
Founder	22 %
FFF & BBAA (19)	39 %
Industrial Investors (3)	39 %

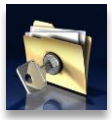


Drug Repositioning or Reprofiling



SOM Biotech's Business Model and Pipeline






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

AREA	PRODUCT	INDICATION	DISCOVERY	In Vitro VALIDATION	In Vivo VALIDATION	PHASE I*	PHASE II	STATUS
METABOLIC ORPHAN	SOM0226	*TTR Amyloidosis*						Phase IIa finished
NEUROLOGY ORPHAN	SOM3355	*Huntington*						Phase IIa to be started Q4-2015
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*						Hit to Lead program

* As repositioned compounds, Phase I can be skipped

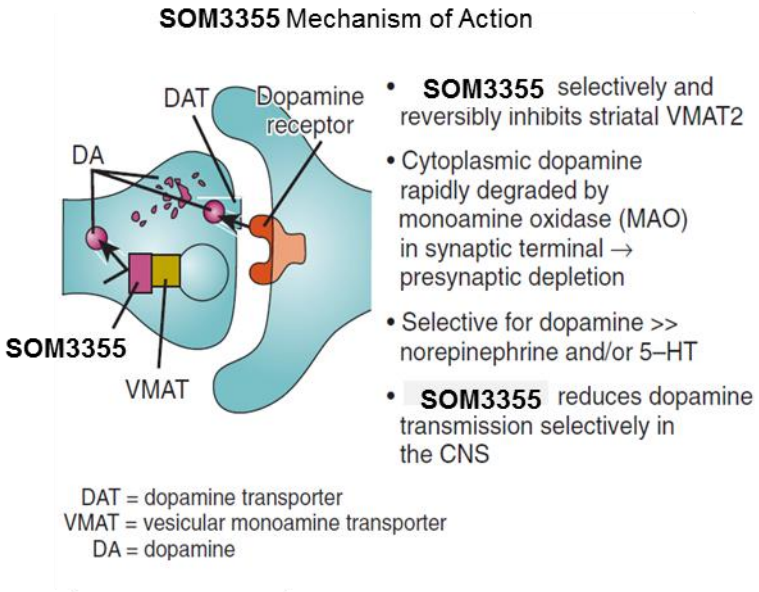
2. The product

SOM3355: a repositioned drug for HUNTINGTON's DISEASE and other related hyperkinetic movement disorders

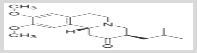
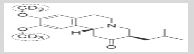
Condition	Disease characteristics	Prevalence	Worldwide potential sales forecast/year
Huntington's disease	• Mutation of the Htt gene • Expanded polyglutamine tract	**2.71/100,000**	**USD 250 M**
Tourette's syndrome	• Tic disorder; multiple physical motor tics • Dysfunction in cortical & subcortical regions (thalamus, basal ganglia and frontal cortex)	**0.4-1/100**	**USD 170.8 M**
Tardive dyskinesia	• Involuntary, repetitive body movements • Results primarily from neuroleptic-induced DA supersensitivity in the nigrostriatal pathway	**20/100 (Wide range of estimations)**	**TBD**
Hemiballism	• Decrease in activity of the subthalamic nucleus of the basal ganglia • Decreased suppression of undesired movements	**TBD**	**TBD**

Mechanism of Action

It has been demonstrated that SOM3355 inhibits striatal **Vesicular Monoamine Transporter-2 (VMAT-2 inhibitor)** and ultimately, it reduces dopamine transmission selectively in the CNS.



Tetrabenazine: Available approved symptomatic treatment (VMAT-2 Inhibitor).

SOM3355 vs. Competitors	SOM3355	Tetrabenazine	SD-809 Auspex's deutered tetrabenazine
MoA/Administration	VMAT2-inhibitor / Oral	VMAT2-inhibitor / Oral	VMAT2-inhibitor / Oral
Chemical structure	Totally Different		
Safety: General aspects	**+++** No significant SAEs reported for its primary indication	**–** **Black Box reported by FDA** (Parkinsonism, suicidal thoughts and Neurol. Malignant Syndrome)	**+/-** Short-term safety profile (low rates of adverse events) **Long-term safety unknown**
Safety: Neurological afflictions depression, psychosis and aggressive behavior	**+++** Not contraindicated for HD patients presenting these neurological afflictions	**–** American Academy of Neurology does not recommend TBZ when these neurological afflictions are present.	**–** It is suggested not to be used in HD patients with these neurological afflictions during clinical phases*
Safety : Hepatic function	**+++** Not contraindicated	**–** Contraindicated in patients with hepatic impairment.	**–** Contraindicated in patients with hepatic impairment.
Dosage	**++** Once-or twice-daily dosing	**–** 3 times daily with High Cmax	**+** Twice daily dosing expected
Other potential indications	**++** Tourette's Syndrome's, Tardive Dyskinesia and Hemiballism	**++** Tourette's Syndrome's, Tardive Dyskinesia and Hemiballism in some countries	**++** Potentially active for Tourette's Syndrome's, Tardive Dyskinesia and Hemiballism
Price	**++** 2.700€/patient/year est. (wide margin of negotiation)	**+++** 1.300€/patient/year	**–** High price expected (reimbursement problems)

* Large HD population (with psychiatric and neurological afflictions) excluded from clinical trials.

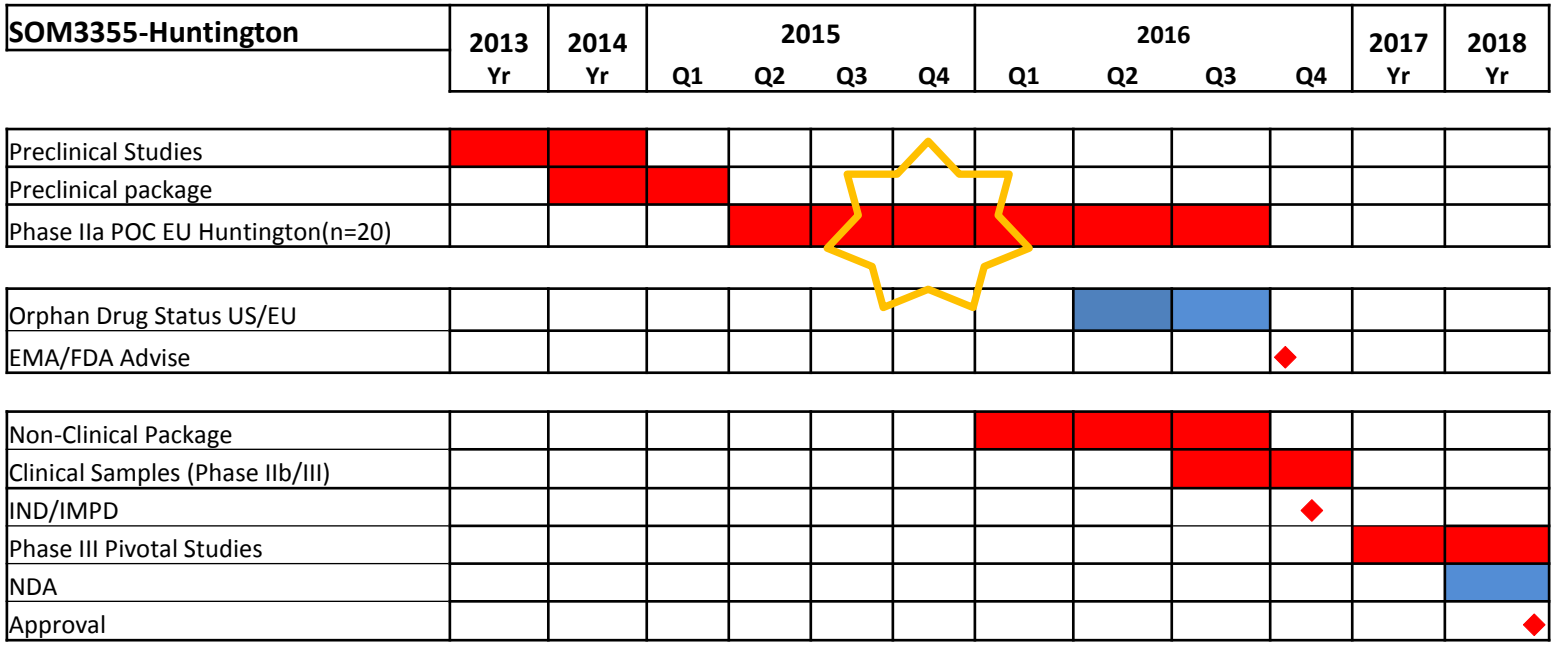
Program's pipeline

In silico
Top 100 list
2012

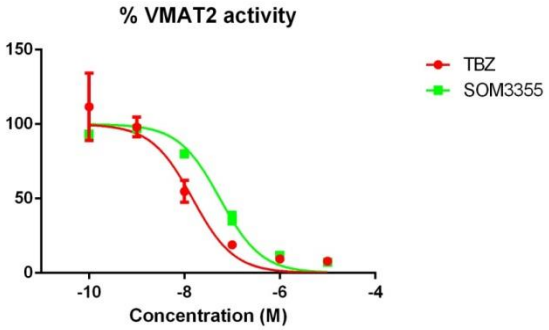
In vitro
VMAT2 inhibition assay
2013

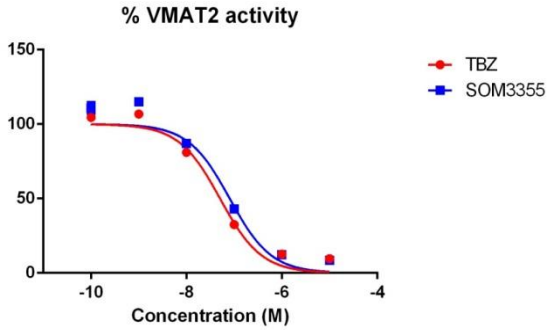
IP
EP patent filed
'Therapeutic agents for use in the prophylaxis and/or treatment of hyperkinetic movement disorders'
JUN 2013

In vivo
Quantification of SOM3355 brain levels after oral administration
2013-2014



Results Summary





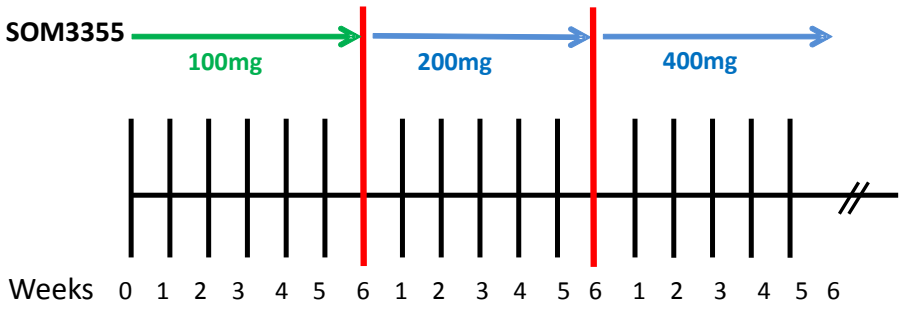
Product code	IC50
Tetrabenazine	36.7 nM
SOM3355	60.3 nM

Product code	IC50
Tetrabenazine	10.5 nM
SOM3355	42.8 nM

SOM3355 Dose	Brain/plasma concentration ratio*
50 mg/kg	5.45
100 mg/kg	12.94

* ng SOM3355/gram of brain in relation to ng SOM3355/gram of plasma

PoC Study scheme. UHDRS assessment



IPR Protection

- **European patent application filed on 19th June 2013**

  Application patent number: EP 13 38 2230

- **Extended European Search Report received on Dec 2013**

  Full revision of the references found by the examiner

- **PCT filling on June 2014:** in the process of re-writing the document according to EESR
- **Patent publication: 24th Dec 2014**
- **National Stage entry expected on Dec 2015**

Pitfalls & Risks to be considered

- Generic drug competitor in the market (with bad safety profile and difficult compliance).
- SOM3355's MoA not novel (VMAT-2 largely used and effective).
- Orphan drug designation not yet obtained (to be filed when human data is available).

3. Partnering Opportunities

- SOM Biotech's business model is based in **licensing out** its current portfolio programs. After Patent of New Use is filed and before or after Human PoC is performed.
- **Joint-venture agreements** for the development of repositioned drugs is also a common partnering opportunity.
- Also interested in **in licensing** repositioning programs aligned with SOM's pipeline.

SOM's mission is to provide worldwide repositioned drug access by **discovering, patenting, developing and licensing** the application of already available drugs for their development and commercial use in unknown indications.

Dr. Santiago Esteva

+34 934 020 150/969

info@sombiotech.com

Parc Científic de Barcelona
Baldiri Reixac, 4, Torre I
08028 Barcelona. Spain
www.sombiotech.com