

CoNCERT Pharmaceuticals, Inc.

Corporate Headquarters:

99 Hayden Avenue
Suite 500
Lexington, MA 02421
Tel: 781.860.0045

Founded: April 2006

Nasdaq Listed: CNCE

Co-Founders:

Richard Aldrich
Roger Tung
Christoph Westphal

Employees:

66

Q2 2017 Financial Results

Cash & Investments: \$103.4 M*

Revenue: \$15,000

Operating Expenses: 13.0 M

Shares Outstanding: 22.6 M

* Includes \$30 M venture debt financing

In July 2017, Concert received \$160 M from Vertex Pharmaceuticals under the CTP-656 asset purchase agreement

Investor and Media Contact:

Justine E. Koenigsberg
Vice President, Corporate
Communications and Investor Relations
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Concert Pharmaceuticals is a clinical stage biotechnology company pioneering the use of deuterium in medicinal chemistry. Concert applies its proprietary DCE Platform[®] (deuterated chemical entity) to approved drugs that we believe can be improved with deuterium substitution to provide better pharmacokinetic or metabolic properties and thereby enhance clinical safety, tolerability or efficacy. Because Concert's new chemical entities are deuterated versions of existing and validated drugs, they are expected to have a high probability of clinical success and achieve early clinical proof-of-concept. By leveraging the large body of knowledge on known drugs, Concert focuses its efforts on valuable opportunities where deuterium modification may provide an important medical benefit.

Potential advantages of our selective deuteration include:

- *Improved metabolic profile.* We have selectively deuterated compounds and compounds produced by metabolism of other compounds, which are called metabolites, to improve their metabolic profiles by reducing the formation of toxic or reactive metabolites or by increasing the formation of desired, active metabolites relative to the corresponding non-deuterated compound. The improved metabolic profile may potentially reduce or eliminate unwanted side effects or undesirable drug interactions.
- *Improved oral bioavailability.* We have selectively deuterated compounds to reduce the extent of undesired metabolism in the wall of the intestines and in the liver, referred to as first-pass metabolism. This resulted in a larger percentage of unmetabolized drug reaching the target site of action. Deuterated compounds with improved bioavailability may be active at lower doses.
- *Increased half-life.* We have selectively deuterated compounds to prolong their pharmacokinetic profile, which is an increase in the half-life of the compound in the body. This may decrease the number of doses that a patient is required to take per day or provide more consistent exposure of the compound in comparison to the corresponding non-deuterated compound.

Selective replacement of specific hydrogen atoms with deuterium creates new compounds that retain the pharmacological effectiveness of the validated drug molecules but, in select cases, improve metabolic profiles. Deuterium is a safe, non-radioactive relative of hydrogen that can be isolated from sea water and has been used extensively in human metabolic and clinical studies. The average adult human body contains 1-2⁺ grams of deuterium due to its general abundance in nature.

Pipeline of Novel Deuterated Candidates

Product Candidate	Lead Indication(s)	Phase I	Phase 2	Phase 3	Market	Worldwide Rights
CTP-543 <small>Deuterated ruxolitinib</small>	Alopecia Areata	▶				CoNCERT Pharmaceuticals Inc.
	Undisclosed Autoimmune	▶				
AVP-786 <small>Deuterated dextromethorphan</small>	Alzheimer's Agitation	▶				AVANIR pharmaceuticals Otsuka
	Neurologic/Psychiatric Indications	▶				
CTP-730 <small>Deuterated epremilast</small>	Inflammatory Diseases	▶				Celgene
JZP-386 <small>Deuterated sodium oxybate</small>	Narcolepsy	▶				Jazz Pharmaceuticals

Management Team:

Roger Tung, Ph.D.
Co-Founder, President and CEO

James Cassella
Chief Development Officer

Lynette Herscha
General Counsel

Nancy Stuart
Chief Operating Officer

Virginia Braman
Vice President, Clinical Development

Christine Boisclair
Vice President, Regulatory Affairs

Justine Koenigsberg
Vice President, Corporate Communications and
Investor Relations

Julie Liu, Ph.D.
Vice President, Research and Develop-
ment Management

Amanda Wagner
Vice President, Business Development and
Product Strategy

Scott Weintraub
Vice President, Commercial Product Strategy

Board of Directors:

Richard Aldrich
Chairman of the Board; Co-founder and
Partner, Longwood Founders Fund

Thomas G. Auchincloss, Jr.
Managing Partner, Counterpoint Trading
Company

Ronald Barrett, Ph.D.
Former Chief Executive Officer, XenoPort

Meghan FitzGerald
Partner, L1 Health LLC

Peter Barton Hutt
Senior Counsel, Covington and Burling;
Former Chief Counsel, FDA

Wilf Jaeger, M.D.
Partner and Founder,
Three Arch Partners

Roger Tung, Ph.D.
President and CEO
Concert Pharmaceuticals, Inc.

Christine van Heek
Former Corporate Officer and President,
Therapeutics Division
Genzyme Corporation

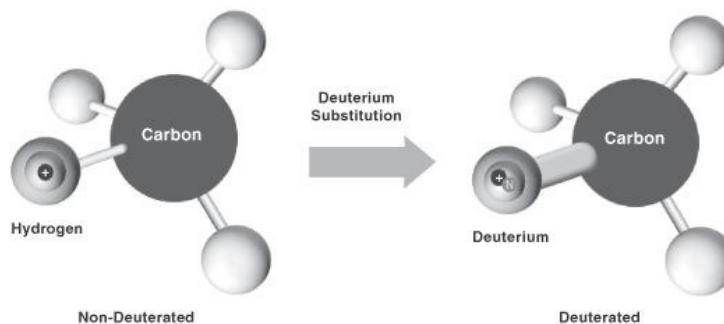
Wendell Wierenga, Ph.D.
Former Executive Vice President, R&D,
Santarus, Inc.

DCE Platform®

Selective incorporation of deuterium in place of hydrogen has the unique benefit of retaining the biochemical potency and selectivity of physiologically active compounds while, in select instances, modifying their metabolic properties to substantially alter their overall therapeutic profile. By starting from compounds with well-defined human pharmacological effects such as approved drugs, deuterium chemistry offers a unique, conceptually straightforward approach to creating significantly differentiated, patentable new medicines that can address important unmet medical needs.

Deuterium

Deuterium is a safe, naturally-occurring relative of hydrogen. Deuterium possesses physicochemical properties that are very similar to those of hydrogen, but its atomic mass is double that of hydrogen. Because of its increased mass, deuterium forms significantly stronger bonds with carbon. Many drugs are metabolized by pathways that involve scission of carbon-hydrogen bonds. Concert exploits the strong deuterium-carbon bonds to improve metabolic profiles, improve oral bioavailability and increase half-life. While deuterium chemistry has a long history of use in the clinical setting – primarily to study bioavailability and drug metabolism. Concert is the first company solely dedicated to the use of deuterium as a drug platform with the goal of creating novel medicines that address commercially important needs.



Broad Patent Estate and Intellectual Property

We protect our product candidates through the use of patents, trade secrets and careful monitoring of our proprietary know-how. Our patent portfolios are wholly owned by us. They include issued patents or patent applications that claim deuterated analogs of more than 90 non-deuterated drugs and drug candidates. In early 2009, the first patents were granted to Concert by the U.S. Patent and Trademark Office (USPTO) with claims to novel compositions of matter. The DCE Platform® has to date resulted in 104 issued US patents and additional notices of allowance. In addition to its young patent estate, Concert also has industry-leading expertise and know-how for developing, optimizing, analyzing, and manufacturing deuterium drug compounds.