



# **Veloxis Pharmaceuticals A/S**

## **2013 Annual Report**

### **Investor Conference**

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# Forward-Looking Statements

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This presentation contains forward-looking statements. All statements other than statements of historical facts included in this presentation are forward-looking statements that are subject to certain risks, trends and uncertainties that could cause actual results and achievements to differ materially from those expressed in such statements. These risks, trends and uncertainties are in some instances beyond our control.

Words such as “anticipate,” “believe,” “estimate,” “expect,” “intend,” “plan,” “will” and other similar expressions identify forward-looking statements, although not all forward-looking statements contain these identifying words. In particular, any statements regarding clinical trial results and potential regulatory approval for Envarsus® (formerly LCP-Tacro™) are considered forward-looking statements. These forward-looking statements involve substantial risks and uncertainties and are based on our assessment and interpretation of the currently available data and information, current expectations, assumptions, estimates and projections about our business and the biopharmaceutical and specialty pharmaceutical industries in which we operate.

Important factors that may affect our ability to achieve the matters addressed in these forward-looking statements include, but are not limited to whether the results of our Phase 3 clinical trials of Envarsus® meet the predetermined endpoints for such trial; our ability to complete the development of, obtain regulatory approval for, and commercialize, Envarsus®; our ability to hire and retain personnel in a competitive industry; our reliance on third parties to manufacture Envarsus® and to conduct clinical trials for Envarsus®; competition from existing therapies and therapies that are currently under development, including Prograf® (tacrolimus), Advagraf®/Astagraf® (tacrolimus), and Nulojix® (belatacept); whether we are able to obtain additional financing, if needed; risks of maintaining protection for our intellectual property; risks of an adverse determination in intellectual property litigation; and risks associated with stringent government regulation of the biopharmaceutical industry.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements, which speak only as of the date hereof. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make. We do not have a policy of updating or revising forward-looking statements and, except as required by law, assume no obligation to update any forward-looking statements.

# Agenda

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- 2013 Key Accomplishments
- Envarsus<sup>®</sup> Status Update
- Financials FY 2013
- Summary

# 2013 Key Accomplishments

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- ✓ New Drug Application (NDA) submitted to the U.S. Food and Drug Administration (FDA) seeking approval for the marketing and sale of Envarsus® for the prevention of organ rejection in kidney transplant recipients
- ✓ Envarsus® granted Orphan Drug Status by FDA
- ✓ Marketing Authorization Application (MAA) for Envarsus® submitted to and accepted for review by the European Medicines Agency (EMA)
- ✓ Positive one-year results from de novo pivotal study 3002
- ✓ Positive results from STRATO tremor study
- ✓ ASERTAA (A Study of Extended Release Tacrolimus in African-Americans) Phase IIIb study of Envarsus® in kidney transplant recipients initiated

# Envarsus<sup>®</sup> Update



# Envarsus® Update – Regulatory

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- US NDA submitted to the FDA December 30, 2013
  - For the prevention of organ rejection in kidney transplant recipients
  - Based on Phase I, II and Studies 3001 and 3002
  - PDUFA (Prescription Drug User Fee Act) action date anticipated to be communicated by the FDA in March 2014
  - Orphan Drug Designation granted by the FDA in December 2013
- European MAA Filing submitted April 29, 2013
  - Acceptance of Filing received May 2013
  - Interactions ongoing with EMA as review process proceeds
  - Anticipate EMA decision in 2014

# Envarsus® Update – Clinical

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- Pivotal blinded phase III Study 3002 in de novo kidney transplant patients
  - Successfully completed Year 1
    - Study achieved its primary efficacy end-point of non-inferiority to Prograf, with rapid attainment of therapeutic tacrolimus blood levels
    - Similar safety and tolerability profile demonstrated
    - Once-daily dosing with lower dose requirements at steady state
    - Data presented at the European Society of Organ Transplantation in September 2013
  - Year 2 Extension on-going
- STRATO Phase IIIb/IV study in Tremor patients
  - Designed to assess potential of once-daily Envarsus® to reduce tremor in patients who have this side effect while receiving twice-daily tacrolimus
  - Study completed
    - Demonstrated potential improvement in tremor symptoms following conversion to Envarsus® and improvement in quality of life measures
    - Data presented at American Transplant Congress in May 2013
- ASERTAA (A Study of Extended Release Tacrolimus in African-Americans) Initiated
  - Phase IIIb study of Envarsus® in African-American kidney transplant recipients



# Envarsus® Update – Commercial

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- United States
  - Commercial preparation activities underway
  - US commercial infrastructure build on course to be ready for upcoming US launch
  - Phase IV planning activities progressing to initiate further differentiation studies
- Europe
  - Commercial planning and launch preparations proceeding via partner Chiesi in close collaboration with Veloxis
- Rest Of World
  - Veloxis exploring opportunities to partner Envarsus® in other geographies



# Financial results



# Annual Report 2013

MDKK	Outlook 2013	Full year		Outlook 2014
		2013	2012	
Revenue		<b>38,2</b>	6,9	
Research and development		<b>(146,5)</b>	(210,7)	
General and administration		<b>(27,8)</b>	(36,9)	
Restructuring cost		-	(21,5)	
Operating loss	(160) - (190)	<b>(136,1)</b>	(262,2)	(60) - (90)
Net loss	(160) - (190)	<b>(139,3)</b>	(262,7)	(55) - (85)
Cash position year-end	310 - 340	<b>328,7</b>	496,8	230 - 270

- Results better than expected driven by the orphan drug designation
- Cash position in line with expectation

# Summary



# Summary

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- Envarsus<sup>®</sup> remains on target
  - US and EU Regulatory Filings under review by FDA and EMA
  - FDA and EMA Regulatory Actions anticipated during 2014
  - Orphan Drug designation achieved
- Commercial opportunity attractive and commercial planning progressing
  - US Commercial infrastructure build and launch planning on track
  - European partnership with Chiesi in place and operating well
- Financing in place to support development, differentiation and commercialization activities through initial Envarsus<sup>®</sup> launch

**Q & A**

**Thank you for your attention!**

