



## **Corporate Overview: Non-Confidential** January 2023

## **CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS**

This presentation contains forward-looking statements, which are based on our management's current beliefs, expectations and assumptions about future events, conditions and results and on information currently available to us.

In some cases, you can identify forward-looking statements by terminology such as "anticipate," "believe," "could," "estimate," "expects," "intend," "may," "plan," "potential," "predict," "project," "should," "will," "would" or the negative or plural of those terms, and similar expressions intended to identify statements about the future, although not all forward-looking statements contain these words. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements.

Any statements in this presentation about our expectations, beliefs, plans, objectives, assumptions or future events or performance are not historical facts and are forward-looking statements. These forward-looking statements include, but are not limited to, statements concerning the following:

- the impact of the coronavirus pandemic on our business;
- any statements of the plans, strategies and objectives of management for our operations;
- any statements of plans to develop and commercialize additional products;
- any statements concerning the attraction and retention of highly qualified personnel;
- any statements concerning the ability to protect and enhance our products and intellectual property;
- any statements concerning developments and projections relating to our competitors or industry;
- any statements concerning our financial performance;
- any statements regarding expectations concerning our relationships and actions with third parties; and
- future regulatory, judicial and legislative changes in our industry.

You should refer to "Risk Factors" in (i) Item 3.D. to our Annual Report on Form 20-F filed with the SEC on March 29, 2021 and (ii) Exhibit 99.2 to the Report of Foreign Private Issuer on Form 6-K filed with the SEC on August 13, 2021, for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. Given these risks, uncertainties and other factors, many of which are beyond our control, we cannot assure you that the forward-looking statements in this presentation will prove to be accurate, and you should not place undue reliance on these forward-looking statements. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame, or at all.

We qualify all of our forward-looking statements by these cautionary statements.

This presentation may contain market data and industry forecasts that were obtained from industry publications. These data involve a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. While we believe the market position, market opportunity and market size information included in this presentation is generally reliable, such information is inherently imprecise.

Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to revise any forward-looking statements to reflect events or developments occurring after the date of this presentation, even if new information becomes available in the future.

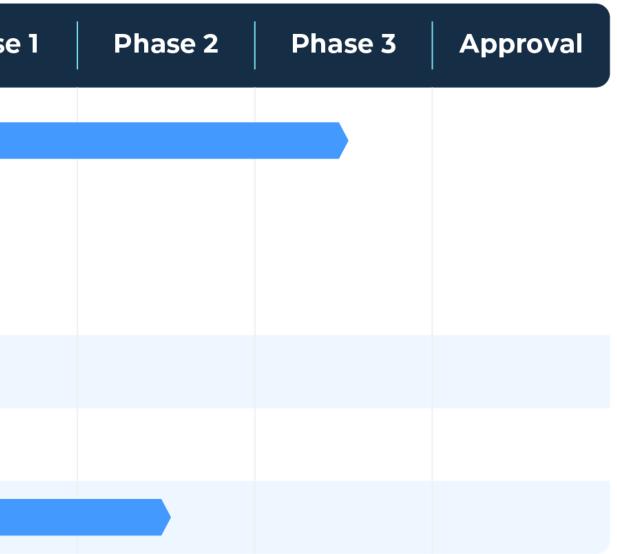


## **Investment Highlights**



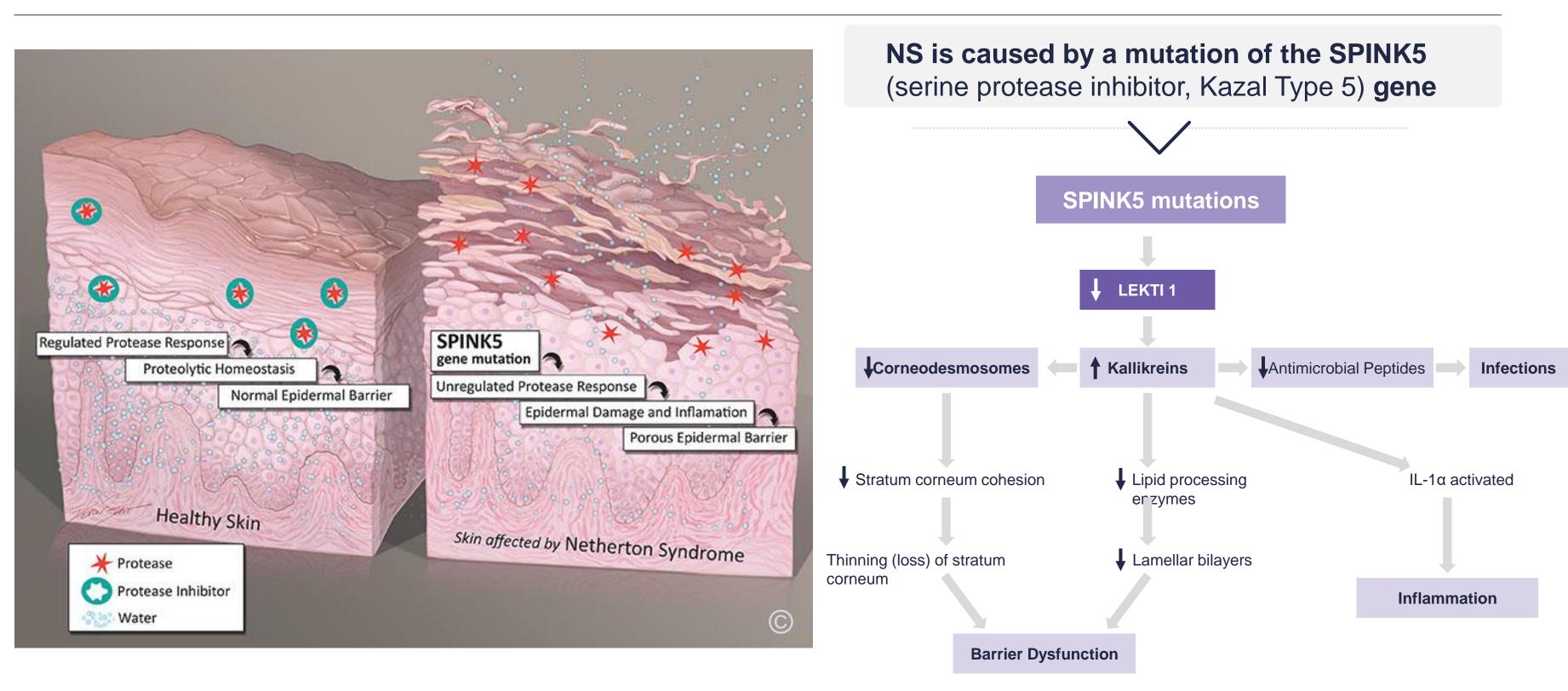
Product Candidate	Indication	Pre-Clinical	Phase
QRX003	<ul> <li>Netherton Syndrome</li> <li>Peeling Skin Syndrome</li> <li>SAM Syndrome</li> <li>Palmoplantar Keratoderma</li> </ul>		
QRX008	<ul> <li>Scleroderma</li> </ul>		
QRX007	<ul> <li>Netherton Syndrome</li> </ul>		
QRX004	• EB		





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## **Netherton Syndrome (NS)**





## **Netherton Syndrome**







## Daily Treatment for the

remainder of the patient's life

- Devastating genetic disease
- Form of Ichthyosis
- Patients suffer from multiple severe issues:
- Infections, allergies, asthma, skin cancer,
- pruritis, warts
- Can be hospitalized on multiple occasions annually
- Environmental toxins, allergens and other micro-organisms can pass through virtually unhindered
- Patients often need to coat their whole body with a moisturizer 5-6 times daily



## **New Treatments Needed**



#### Current options are limited to alleviating some symptoms:

- > Moisturizers typically used for skin barrier repair, such as those containing lanolin and petrolatum, can cause further skin damage to NS patients due to friction or high shear forces on application or removal
- > Topical steroids have been shown to further reduce skin thickness by up to 70% and have led to Cushing Syndrome
- > Other topical treatments, such as calcineurin inhibitors, can lead to dangerously high systemic blood levels due to the defective skin barrier
- > Systemic biologics can provide relief from pruritis but are expensive and not reimbursed

## QRX003 on track to becoming first approved treatment for NS



## **QRX003** Targets the Vicious Circle of Skin Inflammation and Barrier **Disruption**

## Serine Protease Inhibition Plus Anti-inflammatory Activity in the Invisicare Technology

#### Serine Protease Inhibitor/Antiinflammatory

- Targets the KLK5, KLK7 and KLK14 kallikreins that are responsible for excess skin shedding
- Potent anti-inflammatory
- Adequately penetrates the skin but is not absorbed systemically

#### Invisicare DeliveryTechnology

- Immediate protection against **TEWL** and environmental agents
- Moisturizes and protects skin
- Patented polymer delivery system
- **Topical lotion**



#### **QRX003**

Active ingredient replaces missing LEKTI protein, enabling the skin barrier to be repaired. Moisturizes and protects skin.

## **Clinical and Regulatory Pathway**

### **Clear Clinical Path Forward based on FDA Feedback**

- > Approximately 20 Subjects may be sufficient for Approval
- > QRX003 Qualifies for One or More Expedited Approval Pathways
- FDA Recommended Assessing 5 Different Endpoints, Including Composite Endpoints of Investigator and Patient Data
- Lowered Requirements for Achieving a Successful Clinical Outcome

**Positive Scientific Advice Received from EMA** 

**Two Clinical Trials Underway in NS Patients** 

Targeting approval in US and EU in 2024





## **Clinical and Regulatory Pathway: Study 1**

- Single Pivotal Trial
  - All patients must fully wash out of off-label systemic therapy prior to entry into study
- Part A Initiated
  - Double blind 3 arm study in 18 adult NS patients
  - Two doses of QRX003 being tested versus placebo
  - Test materials applied to pre-specified areas of body, once-daily over 12-week period
- Part B to Start in Q3/4 2023
  - Double blind 2 arm study in 30 NS patients
  - Single dose of QRX003 versus placebo
  - Study will recruit both adult and pediatric patients
  - Same study format as Part A



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## **Clinical and Regulatory Pathway: Study 2**

- Open Label Trial
  - All patients must be currently treated with off-label systemic therapy
  - Patients must remain on systemic therapy throughout the 12-week study
- Initiated December 2022.
- Single arm study in 10 adult NS patients
- No placebo control
- Same sites and investigators as Study 1
- QRX003 being assessed as adjuvant treatment
- Clinical endpoints the same as in Study 1



## **QRX003: Additional Information**



**QRX003** formulation is fully developed and has been manufactured at commercial scale



**GMP** supplier of API has been established

CMO's have capacity to supply full commercial requirements





Strong KOL support from leading Netherton experts including Dr. **Amy Paller and Professor Alan Irvine** 

#### 

Working closely with supporting foundations and will have access to patient registries







Anticipate applying for **Orphan Drug status and Pediatric Rare Disease Designation for QRX003** in 2023.

**8 Individual Distribution Partnerships established** in Australia, New Zealand, Middle East, **Central and Eastern** Europe, Turkey, CIS, LATAM, China, Hong **Kong and Canada** 

## **Attractive Commercial Opportunity**

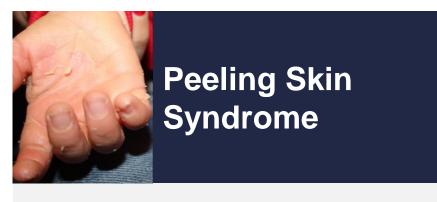




## **Small, compact sales force** will effectively detail product in both US and EU

**Upside sales potential** outside of US and EU. Eight partnerships established covering almost 60 countries. Potential to participate in Early Access programs ahead of regulatory approval

## **QRX003 For Additional Rare Skin Disorders**



- ≻ <1/1000000
- Caused by mutations in the TGM5 gene
- Causes painless peeling of the top layer of skin
- Most apparent on the hands and feet



Severe dermatitis, multiple allergies, and metabolic wasting (SAM)

Caused by mutations in the desmoglein 1 gene (DSG1)

Currently no approved treatments for these disorders. Initial clinical testing to commence in 2023.





#### Palmoplantar Keratoderma

- ➤ 4.4 cases per 100,000
- Causes Thickening of the skin on the hands and feet
- Can be acquired or inherited

## **QRX008 for Scleroderma**

- In-licensed from Queensland University of technology (QUT), Australia
- No currently approved treatments for scleroderma, a rare and sometimes fatal autoimmune disease
- Caused by over production of collagen which results in hardening of the skin and connective tissue
- Focus is on investigating small molecule inhibition of the VCAM-1: VL-4 interaction
- There is an established genetic and clinical link for VCAM1 in scleroderma and the pivotal role VL-4 plays in controlling immune cell migration into inflamed tissue
- Therefore, the VCAM-1:VL-4 interaction is an attractive target for therapeutic intervention in scleroderma.
- Proof of concept has already been established in a mouse model
- Additional studies underway to select a candidate for clinical testing which will begin 2H 2023

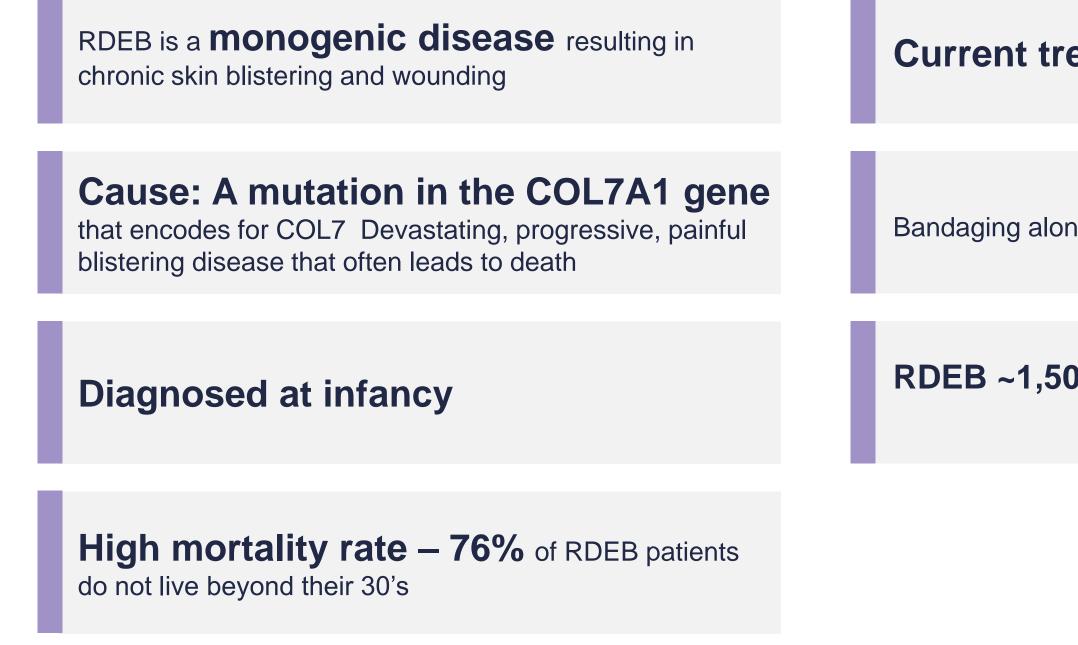


## **QRX007** for Netherton Syndrome

- Also, in-licensed from QUT, Australia
  - Active is a dual domain serine protease inhibitor with proven anti inflammatoryactivity
  - Already in use as a biopharmaceutical (Ulinastatin, Miraclid) for treatment of acute and chronic pancreatitis, Sepsis and toxic epidermal necrolysis Active has achieved low nanomolar inhibitory potencies against the KLK7 and
  - KLK5 kallikreins
  - Drug is a human protein and so is highly unlikely to provoke an immune response
- Pre-clinical program underway at QUT
- Quoin has global rights in return for a mid-single digit royalty on future sales



## **QRX004 For Recessive Dystrophic Epidermolysis Bullosa (RDEB)**





Current treatments only address symptoms

Bandaging alone can exceed \$10,000 per month

RDEB ~1,500 – 2,500 patients in US

## **Clinical and Regulatory Overview**

#### Positive FDA guidance received from Pre-IND filing

#### Program on hold, pending review of market opportunity

#### **Proof of Concept**

28 day, 6-8 patient study in US	Patients act as internal controls	Primary endpoint is proportion of wo healing for patients who receive QI

#### Registration

15-20 patient study with same design as Phase 2 study

Clear precedence for this approach based on competitors experience



ounds with greater than 50% QRX004 vs placebo vehicle

## **Strong Management Team with Proven Track Record of Success**

Name	<b>Position</b>	
Dr. Michael Myers	CEO	elan FUISZ
Denise Carter	COO	
Gordon Dunn	CFO	NewSM

Seasoned executives with over 80 years experience developing products based on drug delivery technologies Proven track record transitioning companies to key inflection points, including mergers, reverse mergers acquisitions and IPOs

Raised over \$250M in private and public markets.







# **THANK YOU!**

# THE THE PHARMACEUTICALS