



Planned Acquisition of Nightstar Therapeutics

Gene Therapy Candidates for Inherited Retinal Disorders

March 4, 2019

Forward looking statements

This presentation contains forward-looking statements, including statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, relating to the potential benefits and results that may be achieved through the proposed acquisition; risks and uncertainties associated with drug development and commercialization; the potential benefits, safety, and efficacy of investigational therapies, including NSR-REP1, NSR-RPGR, NSR-ABCA4, and other potential adeno-associated virus (AAV)-based gene-therapies for inherited retinal disorders; the clinical development program for NSR-REP1 and NSR-RPGR; the anticipated completion and timing of the proposed acquisition; Biogen's strategy and plans; the potential of Biogen's commercial business and pipeline programs, including NSR-REP1, NSR-RPGR, NSR-ABCA4, and other potential AAV-based gene-therapies for inherited retinal disorders; and Biogen's capital allocation and investment strategy. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. These forward-looking statements may be accompanied by such words as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "intend," "may," "plan," "potential," "possible," "will," "would," and other words and terms of similar meaning. You should not place undue reliance on these statements or the scientific data presented.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including: risks that the proposed acquisition will be completed in a timely manner or at all; the possibility that certain closing conditions to the proposed acquisition will not be satisfied; uncertainty as to whether the anticipated benefits of the proposed acquisition can be achieved; the ability of Biogen to successfully integrate Nightstar Therapeutics' (NST) operations and employees; risks of unexpected costs or delays; uncertainty of success in the development and potential commercialization of NSR-REP1, NSR-RPGR, NSR-ABCA4, and other potential AAV-based gene-therapies for inherited retinal disorders, which may be impacted by, among other things, the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis; regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of NSR-REP1, NSR-RPGR, NSR-ABCA4, and other potential AAV-based gene-therapies for inherited retinal disorders; Biogen may encounter other unexpected hurdles, which may be impacted by, among other things, the occurrence of adverse safety events, failure to obtain regulatory approvals in certain jurisdictions, or failure to protect intellectual property and other proprietary rights; the risk that positive results in a clinical trial may not be replicated in subsequent or confirmatory trials or success in early stage clinical trials may not be predictive of results in later stage or large scale clinical trials or trials in other potential indications; product liability claims; and third party collaboration risks. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from our expectations in any forward-looking statement. Investors should consider this cautionary statement, as well as the risks factors identified in our most recent annual or quarterly report and in other reports we have filed with the Securities and Exchange Commission.

These statements are based on our current beliefs and expectations and speak only as of the date of this presentation. We do not undertake any obligation to publicly update any forward-looking statements, whether as a result of new information, future developments, or otherwise.



Additional information

Additional Information and Where to Find It

This communication may be deemed to be solicitation material in respect of the proposed acquisition of NST by Biogen. In connection with the proposed acquisition, NST intends to file relevant materials with the Securities and Exchange Commission (SEC), including a proxy statement in preliminary and definitive form. Holders of NST ordinary shares and American Depositary Shares (ADSs) are urged to read all relevant documents filed with the SEC, including NST's definitive proxy statement, because they will contain important information about the proposed acquisition. Investors and security holders are able to obtain the documents (once available) free of charge at the SEC's web site, <http://www.sec.gov>. Such documents are not currently available.

Participants in Solicitation

Biogen and its directors and executive officers, and NST and its directors and executive officers, may be deemed to be participants in the solicitation of proxies from the holders of NST ordinary shares and ADSs in respect of the proposed acquisition. Information about the directors and executive officers of Biogen and NST is set forth in each company's respective filings with the SEC. Investors may obtain additional information regarding the interest of such participants by reading the proxy statement regarding the proposed acquisition (once available).



Agenda

Introduction

Matt Calistri
VP, Investor Relations

Strategic Overview

Michel Vounatsos
Chief Executive Officer

Scientific Rationale

Michael Ehlers, M.D., Ph.D.
EVP, Research & Development

Deal Terms & Financials

Jeffrey Capello
EVP, Chief Financial Officer

Closing Remarks

Michel Vounatsos
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Available for Q&A

Diana Gallagher, M.D.
VP, Research and Early Development for
Ophthalmology, Pain, and New Indications

Kate Dawson, M.D.
SVP, Therapeutics Development Group



Strategic Overview

Michel Vounatsos
Chief Executive Officer



Planned acquisition of Nightstar Therapeutics

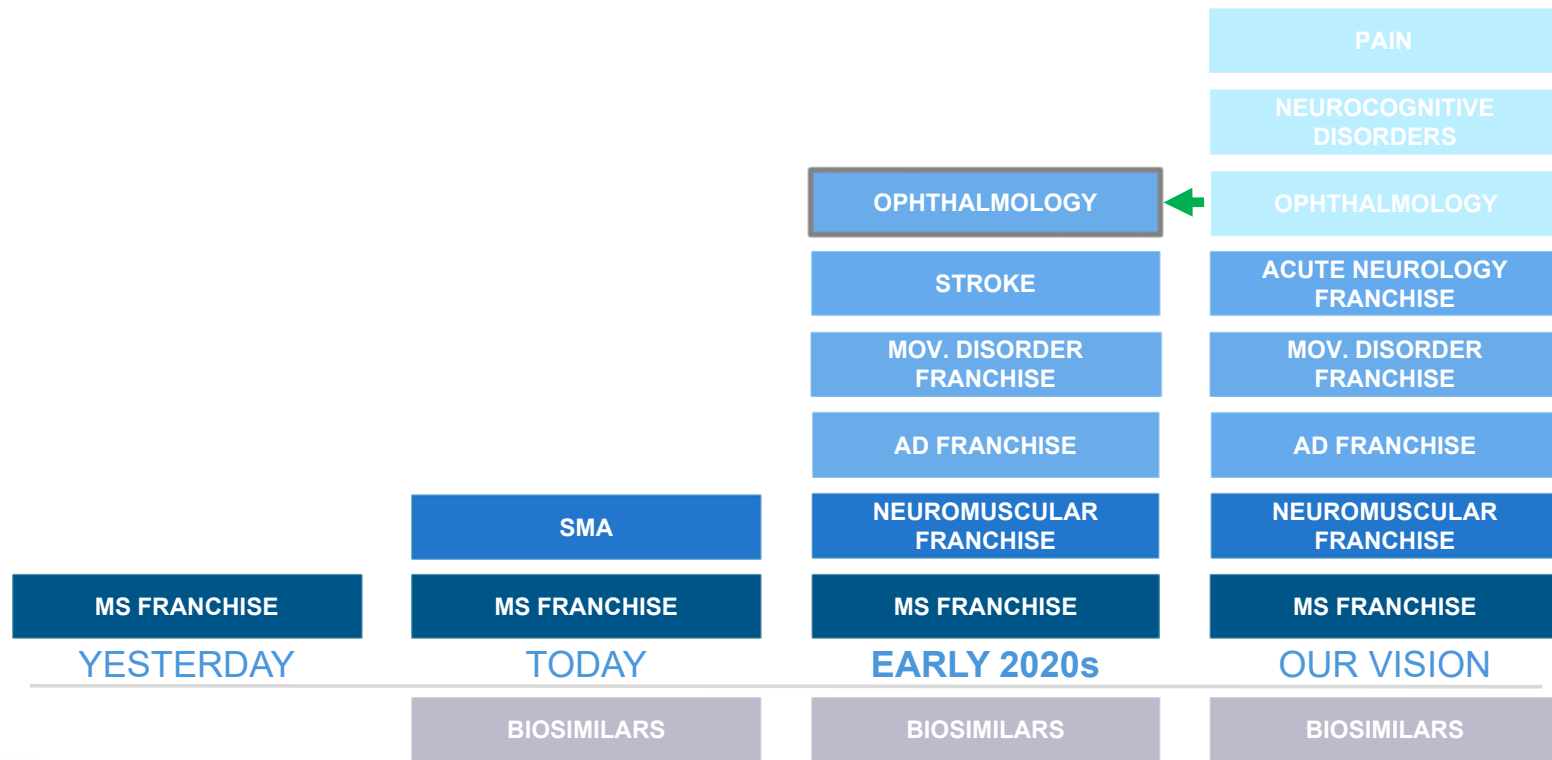
- 1** Strong alignment with Biogen's strategy to develop and expand its **multi-franchise** neuroscience portfolio across **multiple modalities**
- 2** Would **combine Nightstar's expertise** in gene therapy and ophthalmology with **Biogen's core capabilities** in clinical development and rare disease commercialization
- 3** Would give Biogen access to **two potentially first-in-class mid- to late-stage assets** and preclinical programs in a single transaction
- 4** Significant **unmet medical need** in ophthalmic conditions with **no approved treatment options** and potential for **efficient rare disease commercial model**
- 5** **Strong scientific rationale for subretinal delivery** of gene therapy and **encouraging early data** for both clinical assets suggests potentially meaningful benefit versus natural history



Note: The closing of the proposed acquisition is subject to customary closing conditions, including the approval by Nightstar shareholders, issuance of an order by the U.K. Court, and receipt of regulatory approvals.

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Continuing to build a multi-franchise neuroscience portfolio

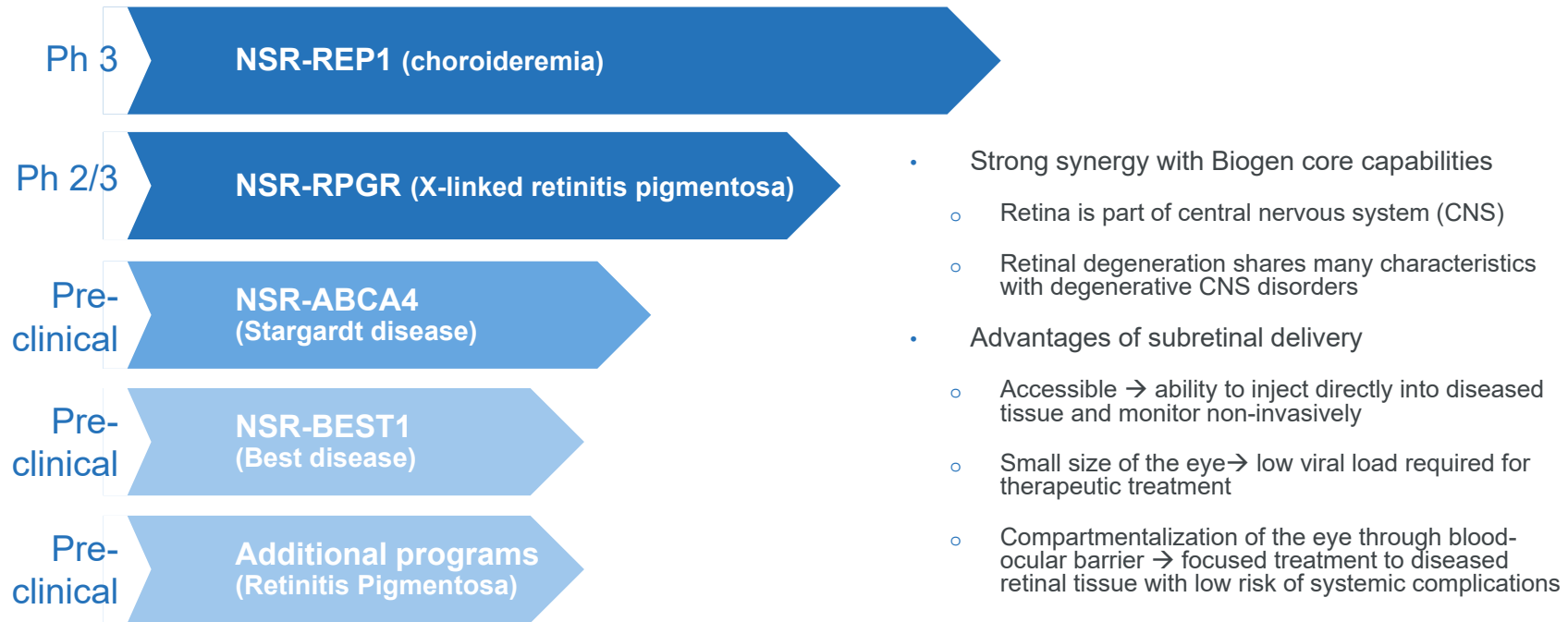


Scientific Rationale

Michael Ehlers, M.D., Ph.D.
EVP, Research &
Development



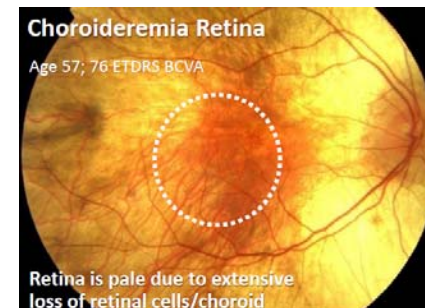
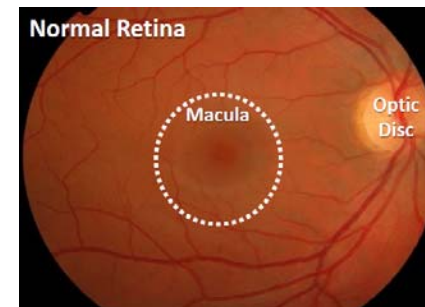
Broad pipeline for inherited retinal disorders



Significant unmet need in choroideremia (CHM)

Rare, degenerative disease that leads to blindness; no treatment options

- Inherited X-linked monogenic retinal disease
 - Affects ~ 1:50,000, primarily males
 - G7 estimated prevalence of ~ 15,000 patients
- Progressive loss of vision leading to blindness
 - Early - Night blindness
 - Mid - Peripheral vision loss
 - Late - Central vision loss
 - Often results in legal blindness by age of 40
- Caused by loss-of-function mutations in CHM gene which encodes Rab escort protein-1 (REP1)
 - REP1 protein involved in membrane trafficking in photoreceptors and retinal pigment epithelium (RPE)
 - Mutations lead to photoreceptor and RPE degeneration, which leads to blindness



Source: MacDonald et al., *Choroideremia*, Feb 2003.

Endpoints in retinal disease clinical trials

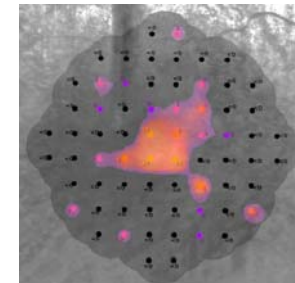
Visual Acuity

- Routinely tested in clinical practice
- Direct functional measure of vision loss/gain



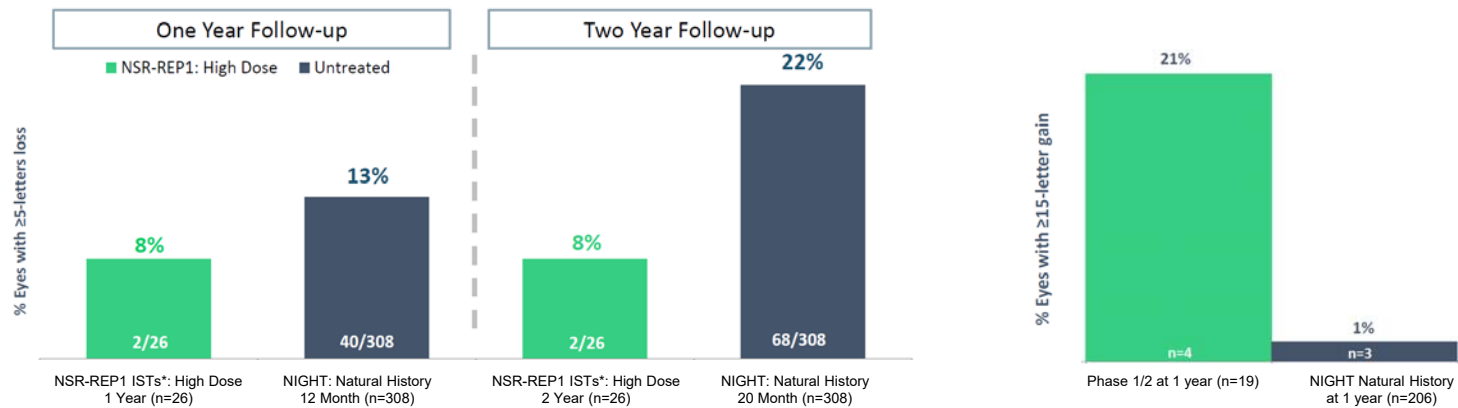
Microperimetry

- Realtime imaging of entire macula
- Automated eye tracking
- Stimulation of same retinal loci across tests/visits
- Higher sensitivity than visual acuity



Compelling Phase 1/2 proof-of-concept data in CHM

Evidence of maintained or improved visual acuity in Phase 1/2 studies



* Based on retrospective analyses of investigator sponsored trials

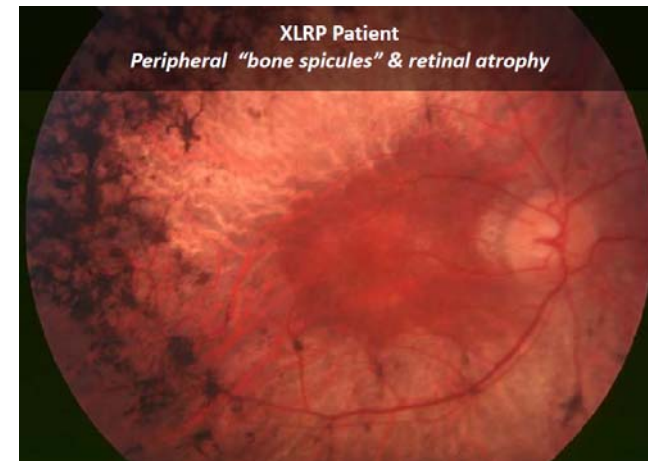
- Current data from 32 treated patients in the completed investigator sponsored trials (ISTs) indicate NSR-REP1 was well tolerated
- Safety profile consistent with vitrectomy
 - Adverse events of varying severity and duration generally resolve within one week: retinal changes, intraocular inflammation and visual disturbances
- 2 Treatment-related Serious Adverse Events
 - 1 Possibly drug-related: intraocular inflammation
 - 1 Procedure-related: gas bubble in surgical tubing



Opportunity in X-linked retinitis pigmentosa (XLRP)

Progressive photoreceptor degeneration that leads to blindness; no treatment options

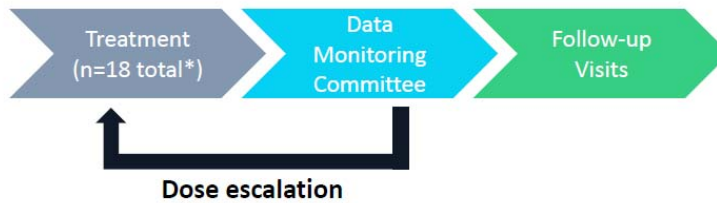
- Rare X-linked monogenic retinal disease
 - Affects ~ 1:40,000, primarily males
 - G7 estimated prevalence of ~ 20,000 patients
- Rapid progressive loss of vision leading to blindness
 - Early - Night blindness
 - Mid - Peripheral vision field constriction
 - Late - Central vision deterioration / loss
 - Median age of legal blindness is 45
- Caused by loss-of-function mutations in retinitis pigmentosa GTPase regulator (RPGR) gene
 - RPGR protein involved in molecular transport between inner and outer segments of photoreceptors
 - Mutations lead to photoreceptor degeneration, which leads to blindness



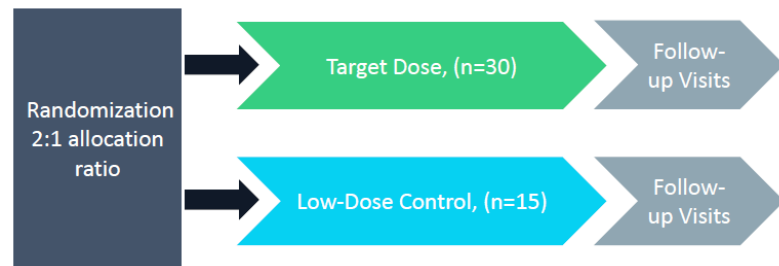
Source: Hamel, *Orphanet Journal of Rare Diseases*, October 2006

XIRIUS clinical trial design for NSR-RPGR in XLRP

Phase 1/2 Dose Escalation Study



Phase 2/3 Expansion Study



Eligibility	Genetically confirmed diagnosis of XLRP Males 18 years and older
Primary Endpoint	Safety: Incidence of dose limiting toxicities & treatment emergent adverse events
Selected Secondary Endpoints	<ul style="list-style-type: none"> Maintenance of vision (BCVA) Changes in microperimetry Changes in SD-OCT Ellipsoid Zone

* 6 cohorts with 3 patients each

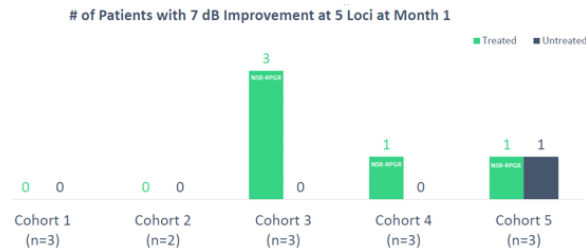
Preliminary data from first 5 cohorts (n=15) presented at EURETINA in 2018

Study initiated Q4 2018, currently enrolling



Early efficacy signal from Phase 1/2 dose escalation study

Increase in central retinal sensitivity



- All patients in Cohort 3 and 2/6 patients in Cohorts 4 & 5 demonstrated early efficacy signal on microperimetry analyses
- Inflammation may have dampened efficacy in higher dose Cohorts 4 and 5
- Early steroid treatment in setting of inflammation was able to restore retinal sensitivity in some patients

Acceptable safety profile

- Current data from 15 treated patients (cohorts 1-5) indicate NSR-RPGR is generally well-tolerated
 - No early discontinuations
 - No dose limiting toxicity observed
 - No serious treatment-related AEs
- Mild transient drug-related inflammation in cohorts 4-5

Potential for two new product launches in early 2020s

Potential first-in-class therapies for diseases with no treatment options

NSR-REP1 for CHM

- Potential to maintain and improve vision of patients with CHM
- Proof-of-concept achieved in Phase 1/2
- Full enrollment in Phase 3 STAR trial expected 1H:2019
- Phase 3 data expected 2H:2020
- FDA regenerative medicine advanced therapy (RMAT) designation
- Orphan drug designation from both FDA and EMA

NSR-RPGR for XLRP

- Potential to maintain and improve vision of patients with XLRP that rapidly progresses to blindness
- Early efficacy signal seen in Phase 1/2 study
- Phase 2/3 dose expansion study currently enrolling
- Expect one additional Phase 3 study to support registration
- Orphan drug designation from EMA

Deal Terms & Financials

Jeffrey Capello
EVP, Chief Financial Officer



Potential for long-term value creation



Financial Details

- Biogen agreed to acquire Nightstar for \$25.50 per share
- Represents a total transaction value of ~ \$800 million on a fully diluted basis, after taking into account expected transaction expenses and anticipated cash at closing
- The closing of the proposed acquisition is subject to customary closing conditions, including the approval by Nightstar shareholders, issuance of an order by U.K. Court, and receipt of regulatory approvals
- Expect to complete the acquisition by mid-year 2019
- Expect the return on invested capital will exceed our weighted average cost of capital in a reasonable time period



Closing Remarks

Michel Vounatsos
Chief Executive Officer



Questions & Answers

