Corporate Overview

October 2023
Forward-Looking Statements

This presentation contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this presentation may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, MHRA, and PMDA, may not grant or may delay approval for our product candidates; the potential that required regulatory inspections may be delayed or not be successful and delay or prevent product approval; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or Pombiliti + Opolda if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. With respect to statements regarding projections of the Company’s revenue and cash position, actual results may differ based on market factors and the Company’s ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2022, and on Form 10-Q for the quarter ended June 30, 2023. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

Non-GAAP Financial Measures

In addition to financial information prepared in accordance with U.S. GAAP, this presentation also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.
A Rare Company

Patient-dedicated, rare disease biotechnology company with sustained double-digit revenue growth, a global commercial infrastructure, and late-stage development capabilities

**Galafold®**
First Oral Precision Medicine for Fabry Disease

**GLOBAL COMMERCIAL ORGANIZATION**

World-class Clinical Development Capabilities

**Gene Therapy Platform**
Leveraging Experience in Protein Engineering & Glycobiology

**Non-GAAP PROFITABILITY**
expected in 2H 2023

**EMPLOYEES**
in 20 Countries

Pombiliti™
(cipaglucosidase alfa-atga)

Opfolda™
(migliustat) 65 mg capsules

Approved in the U.S., EU, and U.K.

14-18%
FY23 Galafold Revenue Growth at CER

GALAFOLD & POMBILITI + OPFOLDA
Cumulative $1.5B-$2B Peak Potential

$266M
Cash as of 6/30/23
2023 Strategic Priorities

1. Double-digit Galafold® revenue growth of 14-18% at CER¹
2. Secure FDA, EMA, and MHRA approvals for Pombiliti™ + Opfolda™
3. Initiate successful global launches of Pombiliti™ + Opfolda™
4. Advance best-in-class, next-generation Fabry and Pompe pipeline programs and capabilities
5. Maintain strong financial position on path to profitability

¹CER: Constant Exchange Rates; 2023 Galafold revenue guidance utilizes actual exchange rate as of December 31, 2022
## Amicus Pipeline

Streamlined rare disease pipeline with focus on Fabry disease and Pompe disease franchises

<table>
<thead>
<tr>
<th>INDICATION</th>
<th>DISCOVERY</th>
<th>PRECLINICAL</th>
<th>PHASE 1/2</th>
<th>PHASE 3</th>
<th>REGULATORY</th>
<th>COMMERCIAL</th>
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<tbody>
<tr>
<td><strong>FABRY FRANCHISE</strong></td>
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<tr>
<td>Galafold® (migalastat)</td>
<td>ODD</td>
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<tr>
<td>Fabry Gene Therapy</td>
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<tr>
<td>Next-Generation Chaperone</td>
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<td><strong>POMPE FRANCHISE</strong></td>
<td>BTD</td>
<td>ODD</td>
<td></td>
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<tr>
<td>Pombiliti™ (cipaglucosidase alfa-atga) + Opolda™ (miglustat)</td>
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<tr>
<td>Pompe Gene Therapy</td>
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<td><strong>OTHER</strong></td>
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<tr>
<td>CLN3 Batten Disease Gene Therapy</td>
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<td>Next-Generation Research Programs</td>
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ODD - Orphan Drug Designation  
BTD - Breakthrough Therapy Designation
Galafold® (migalastat) Continued Growth

Building a leadership position in the treatment of Fabry disease
Global Fabry Market

Global Fabry disease market growth continues to be driven by diagnosis of new patients

- Believed to be significantly underdiagnosed
  - Newborn screening studies suggest Fabry is one of the more prevalent genetic diseases (~1:1,000 to ~1:4,000 incidence)

- In 2021 and 2022, Galafold was the fastest growing Fabry treatment and the greatest contributor to market growth
  - Galafold has led to market expansion with >1,000+ naïve patients treated

Global Fabry Market of ~$1.9B in 2022 and Tracking toward ~$2.6B+ by 2027

<table>
<thead>
<tr>
<th>Year</th>
<th>Sales (millions)</th>
</tr>
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<tbody>
<tr>
<td>2017</td>
<td></td>
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<tr>
<td>2022</td>
<td></td>
</tr>
<tr>
<td>2027E</td>
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</table>

1 Global market measured by reported sales of approved therapies for Fabry disease – 2027 sales projected using ~7% CAGR
Galafold is the first and only approved oral treatment option with a unique mechanism of action for Fabry patients with amenable variants. Galafold is indicated for adults with a confirmed diagnosis of Fabry disease and an amenable variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea, and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit [https://www.amicusrx.com/pi/Galafold.pdf](https://www.amicusrx.com/pi/Galafold.pdf). For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions, and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at [www.ema.europa.eu](http://www.ema.europa.eu).
Galafold Performance

Raising FY23 revenue growth guidance to 14% to 18% at CER

- Global mix of switch (~45%) and previously untreated patients (~55%)³
- Compliance and adherence over 90%+
- Expect non-linear quarterly growth to continue due to uneven ordering patterns and FX fluctuations

FY16 FY17 FY18 FY19 FY20 FY21 FY22 FY23

$5M $37M $91M $182M $261M $306M $329M¹ $375M - $388M²

1H23 reported revenue growth of +13% to $180M with strong operational growth of +16%

¹ FY22 reported revenue growth of +8% to $329M with strong operational growth of +16% at CER – FY22 negative currency impact YoY of ~$26M
² At constant exchange rate (CER)
³ Data on file
Galafold Global Commercial Momentum (as of June 30, 2023)

Strong patient demand and performance against key metrics lay the foundation for continued double-digit growth in 2023.

Sustained Growth in 2023 Driven by:
- Continued penetration into existing markets
- Further uptake in diagnosed untreated population
- Continued geographic expansion and label extensions
- Maintaining compliance and adherence
- Driving reimbursement and access
Pombiliti™ (cipaglucosidase alfa-atga) + Opfolda™ (miglustat)

Potential to establish a new standard of care for people living with Pompe disease
Late-onset Pompe Disease (LOPD) Overview

Late-onset Pompe disease is a rare, debilitating, and life-threatening lysosomal disorder caused by a deficiency of the enzyme acid alpha-glucosidase (GAA).

- Diagnosed at different stages of life, from childhood to adulthood
- Majority of patients on current standard of care decline after ~2 years
- Respiratory failure and loss of motor function are major causes of morbidity and mortality
- Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction
- Symptoms include progressive muscle weakness, particularly skeletal and respiratory muscles, that worsens over time
- ~5,000-10,000 people diagnosed globally; Significant underdiagnosis
- ~$1.2B+ global Pompe ERT sales

1As reported, based on 12 months ended December 31, 2022.
Global Pompe Market

Global Pompe disease market growth continues to be driven by the diagnosis of patients.

- An estimated 3,500-4,000 Pompe patients globally are being treated by ERT.
- Global Pompe Market of ~$1.2B in 2022 and Tracking toward $1.8B+ by 2027.

Global Pompe Market Sales Split FY2022:
- United States: 41%
- Europe: 37%
- Rest of World: 22%

1Global market measured by reported sales of approved therapies for Pompe disease – 2027 sales projected using ~8% CAGR
2As reported FY2022
3Amicus Data on File from Market Mapping
Our scientists created a uniquely glycosylated and highly phosphorylated ERT that enhances targeting to key affected muscles and is co-administered with an enzyme stabilizer.

- Pombiliti + Opfolding is a two-component therapy combining cipaglucosidase alfa-atga, an ERT, with miglustat, an orally administered enzyme stabilizer.
- Pombiliti is expressed in a unique cell line producing a naturally glycosylated and highly phosphorylated enzyme that can be properly processed to its mature form, which are both required for greater lysosomal GAA activity.

1Selvan et al. 2021, J Biol Chem 2021 Jan-Jun;296:100769
ERT: Enzyme Replacement Therapy
Phase 3 OLE of Pombiliti + Opfolda in LOPD

Phase 3 open-label extension study data demonstrate that treatment with Pombiliti + Opfolda up to 2 years was associated with a durable effect, supporting the long-term benefits

ERT-Experienced 6MWD (%): Change from baseline

ERT-Experienced FVC (%): Change from baseline

- ERT-experienced and -naïve patients treated with Pombiliti + Opfolda throughout PROPEL showed durable improvements in % predicted 6MWD that were maintained throughout to week 104
- ERT-experienced and -naïve patients who received alglucosidase alfa/placebo in PROPEL and switched to Pombiliti + Opfolda in the OLE showed stability in % predicted 6MWD throughout the OLE study

ERT-experienced patients treated with Pombiliti + Opfolda throughout PROPEL remained stable, while patients who received alglucosidase alfa/placebo experienced a decline in sitting % predicted FVC that stabilized after switching to Pombiliti + Opfolda in the OLE study
PROPEL Effect Size Analysis in ERT-Experienced Adults

Post hoc analysis shows majority of study parameters demonstrated stabilization or improvement after switching from SoC to Pombiliti + Opfolda

ERT-experienced patients remaining on alg+pbo (n=30) generally showed worsening (d<+0.2) or stability (-0.2≤d<+0.2) across most outcomes, with significant worsening for various lung function assessments and biomarkers.

- Significant improvement was only observed for (unadjusted mean CFBL) QoL: PROMIS–Dyspnea (-1.53).
- Significant worsening was observed for (unadjusted mean CFBL): sitting forced vital capacity (FVC, -4.02%); supine FVC (-2.63%); sitting slow vital capacity (SVC, -6.52%); maximum expiratory pressure (MEP, -3.85%); creatine kinase (CK, 79.6 IU/L); and hexose tetrasaccharide (Hex4, 1.89 mmol/molCr).
- Significant improvement was observed for (unadjusted mean CFBL): 6MWD (16.89 m); 6MWD % predicted (3.20); manual muscle test (MMT) lower extremities (1.63); MMT upper extremities (1.76); MMT overall score (3.38); CK (-118.0 IU/L); Hex4 (-1.69 mmol/molCr); QoL: EuroQol Dimensions-5 Levels Instrument (EQ-5D-5L)–Pain/Discomfort (-0.19); QoL: PROMIS–Fatigue (-1.87); QoL: Subject’s Global Impression of Change (SGIC)–Overall (0.34); QoL: SGIC–Ability to Move Around (0.21); QoL: SGIC–Muscle Function (0.20); QoL: SGIC–Daily Living (0.28); QoL: SGIC–Energy Level (0.33); and QoL: other–Physician’s Global Impression of Change (PGIC)–Overall (0.27).
- Significant worsening was not observed for any assessments.

ERT-experienced patients switching to cipa+mig mostly showed improvement or stability, with significant improvements for various assessments of motor function, muscle strength, biomarker, and global impression of change scales.

- Significant improvement was observed for (unadjusted mean CFBL): 6MWD (16.89 m); 6MWD % predicted (3.20); manual muscle test (MMT) lower extremities (1.63); MMT upper extremities (1.76); MMT overall score (3.38); CK (-118.0 IU/L); Hex4 (-1.69 mmol/molCr); QoL: EuroQol Dimensions-5 Levels Instrument (EQ-5D-5L)–Pain/Discomfort (-0.19); QoL: PROMIS–Fatigue (-1.87); QoL: Subject’s Global Impression of Change (SGIC)–Overall (0.34); QoL: SGIC–Ability to Move Around (0.21); QoL: SGIC–Muscle Function (0.20); QoL: SGIC–Daily Living (0.28); QoL: SGIC–Energy Level (0.33); and QoL: other–Physician’s Global Impression of Change (PGIC)–Overall (0.27).
- Significant worsening was not observed for any assessments.

*Indicates nominal statistical significance; ‡For these endpoints, a negative CFBL value indicated a better result. For visualization purposes, the direction of the results was reversed so that positive CFBL values indicate better results.

GSGC, Gait, Stairs, Gowers’ maneuver, and Chair; LOCF, last observation carried forward; MEP, maximum inspiratory pressure; QMT, quantitative muscle test; SNIP, sniff nasal inspiratory pressure; TUG, timed up and go; VAS, visual analogue scale.
Global Regulatory Status

Pombiliti + Opfolda approved and launched in the three largest Pompe markets in 2023

- Pombiliti® + Opfolda® now approved in the EU
- Pombiliti® + Opfolda® now approved in the U.K.
- Pombiliti™ + Opfolda™ now approved in the U.S.
Ongoing Clinical Studies and Expanded Access Mechanisms

- Ongoing clinical studies in children and adolescents\(^1\) with LOPD and infantile-onset Pompe disease (IOPD)
- Multiple expanded access mechanisms in place
- At time of first regulatory approval, ~200 people living with Pompe disease on Pombiliti + Opfolda across extension studies and expanded access programs
- ~75 centers worldwide currently participating in clinical trials and access programs

\(^1\)Children and adolescents aged 0 to <18 years old
Pombiliti + Opfolda EU Opportunity

EU Pompe market currently represents a sizeable market opportunity of $450M+

- **Strong indication statement:**
  - Pombiliti® (cipaglucosidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser Opfolda® (miglustat) for the treatment of adults with late-onset Pompe disease (acid α glucosidase [GAA] deficiency)

- >1,300 patients are estimated to be treated in Europe\(^1\)
  - ~60 Patients throughout EU currently on Pombiliti + Opfolda, including ~20 in Germany and Austria

- Launch underway in Germany
  - 6 month “free pricing” period and AMNOG reimbursement process
  - First patients dosed and additional patients scheduled to start infusions

\(^1\) Amicus data on file from market mapping
Launch of Pombiliti + Opfolda Underway in the EU

Experienced and passionate rare disease commercial and medical organization supporting early days of launch

Performance
Patient Demand
Initial focus on clinical trial and expanded access patients
First patients dosed; Multiple scheduled for infusion
On-track to transition all trial and expanded access patients in Germany within 90 days

KOL and Patient Outreach
Promotion and Education Efforts
Existing relationships with HCPs at key treatment centers
Engaging top prescribers within first 30 days
Ongoing disease education

Access and Reimbursement
Positive Interactions with Payors
Focus on broad patient access
Country-by-country reimbursement process
Active discussions to demonstrate value
Corporate Outlook

Delivering on our mission for patients and shareholders
Financial Outlook and Path to Profitability

Clear strategy to build our business, advance our portfolio, and achieve profitability

**Sustain Revenue Growth**

- $180.8M 1H 2023 revenue, +16% YoY operational growth
- 2023 Galafold revenue growth guidance of +14-18% YoY at CER

**Secure Approvals of Pombiliti + Opfolda**

- Galafold and Pombiliti + Opfolda expected to drive strong double-digit growth long term

**Deliver on Financial Goals**

- Focused on prudent expense management
- 2023 non-GAAP operating expense guidance of $330M-$350M
- Achieve profitability\(^1\) in 2H 2023

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\(^1\) Based on projections of Amicus non-GAAP Net Income under current operating plans, which includes successful Pombiliti + Opfolda regulatory approvals and continued Galafold growth. We define non-GAAP Net Income as GAAP Net Income excluding the impact of share-based compensation expense, changes in fair value of contingent consideration, depreciation and amortization, acquisition related income (expense), loss on extinguishment of debt, loss on impairment of assets, restructuring charges, and income taxes.
Positioned for Significant Value Growth

Focused on execution and driving sustainable double-digit revenue growth on path to profitability

- Continue to bring Galafold® to as many patients as possible, sustain double-digit operational revenue growth
- Successful launch of Pombiliti + Opfolda for people living with Pompe disease
- Advance next-generation gene therapies in Fabry and Pompe diseases
- Fully leverage global capabilities and infrastructure as a leader in rare diseases
- Achieve non-GAAP profitability in 2H 2023

1 Based on projections of Amicus non-GAAP Net Income under current operating plans, which includes successful Pombiliti + Opfolda regulatory approvals and continued Galafold growth. Non-GAAP Net Income defined as GAAP Net Income excluding the impact of share-based compensation expense, changes in fair value of contingent consideration, loss on impairment of assets, depreciation and amortization, acquisition related income (expense), loss on extinguishment of debt, restructuring charges and income taxes.
True Measure of Success: Impacting the Lives of People Living with Rare Diseases

>350 Patients*

>2,000 Patients*

Thousands of Patients*

YE17

YE22

2023+

*Clinical & commercial; all figures approximate
### Reconciliation of Non-GAAP Financial Measures

(in thousands)

<table>
<thead>
<tr>
<th></th>
<th>Three Months Ended June 30,</th>
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<th>Six Months Ended June 30,</th>
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<td></td>
<td>2023</td>
<td>2022</td>
<td>2023</td>
<td>2022</td>
</tr>
<tr>
<td><strong>Total operating expenses - as reported GAAP</strong></td>
<td>$104,249</td>
<td>$133,147</td>
<td>$221,213</td>
<td>$279,619</td>
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<tr>
<td><strong>Research and development:</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>Stock-based compensation</td>
<td>4,117</td>
<td>4,379</td>
<td>12,607</td>
<td>13,744</td>
</tr>
<tr>
<td><strong>Selling, general and administrative:</strong></td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>Stock-based compensation</td>
<td>12,460</td>
<td>8,084</td>
<td>38,864</td>
<td>29,370</td>
</tr>
<tr>
<td>Loss on impairment of assets</td>
<td>1,134</td>
<td>-</td>
<td>1,134</td>
<td>6,616</td>
</tr>
<tr>
<td>Changes in fair value of contingent consideration payable</td>
<td>337</td>
<td>115</td>
<td>588</td>
<td>(1,073)</td>
</tr>
<tr>
<td>Depreciation and amortization</td>
<td>2,206</td>
<td>1,334</td>
<td>3,463</td>
<td>2,745</td>
</tr>
<tr>
<td><strong>Total operating expense adjustments to reported GAAP</strong></td>
<td><strong>20,254</strong></td>
<td><strong>13,912</strong></td>
<td><strong>56,656</strong></td>
<td><strong>51,402</strong></td>
</tr>
<tr>
<td>Total operating expenses - as adjusted</td>
<td><strong>$83,995</strong></td>
<td><strong>$119,235</strong></td>
<td><strong>$164,557</strong></td>
<td><strong>$228,217</strong></td>
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2023 Environmental, Social, & Governance (ESG) Snapshot

Who We Serve

- Programs we invest in have 3 key characteristics
  - Address a rare genetic disease
  - First-in-class or best-in-class
  - Impart meaningful benefit for patients

Pledge for a Cure

- Designate a portion of product revenue back into R&D for that specific disease until there is a cure.

Pricing PROMISE

- Committed to never raising the annual price of our products more than consumer inflation.

Environmental Management

- Committed to producing transformative medicines for patients while practicing environmental responsibility and adhering to sustainability best practices in our operations.
- Our mission is to drive sustainability with our partners by incorporating environmental and sustainability principles into all our commercial relationships.

- Global Employees: 484
- % Female Employees: 57%
- % Hiring Slate Diversity: 97%

Charitable Giving

- Contributions allocated:
  - $2,288,998 U.S.
  - $954,349 Intl.
- Expanded Access through Feb 2023: 79 patients / 19 countries
- Amicus supported community programs: 22
- Volunteer hours (U.S.): 580

Diversity, Equity, & Inclusion (DEI)

- Pledge to support a more inclusive culture to impact our employees, our communities, and society.
- Goal of maintaining gender diversity and increasing overall diversity throughout our global workforce.

Employee Recruitment, Engagement, & Retention

- Leverage employee capabilities and expertise to provide a culture that drives performance and ultimately attracts, energizes, and retains critical talent.
- Pulse surveys reveal employees feel high personal satisfaction in their job, are proud of their work and what they contribute to the community.

Board of Directors

- Committed to ongoing Board refreshment and diversity of background, gender, skills, and experience:
  - Director Diversity:
    - 3 Female
    - 2 Veteran Status
    - 1 African American
  - 80% Board Independence
  - 60% Overall Board Diversity

Career Development

- Reimagined performance management process to measure the what and the how, rewarding those who role-model our Mission-Focused Behaviors.
Thank you