



Clinical Trial Diversification Better Practices

TOPIC 2: PATIENT ENGAGEMENT

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Version 1.1

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flex· i· ble

'fleksəbəl/

adjective

adjective: **flexible**

1. capable of bending easily without breaking.

"flexible rubber seals"

synonyms: pliable, supple, bendable, pliant, plastic; More

TransCelerate Clinical Trial Diversification Workstream Summary

This slide deck and the materials contained within it were compiled by the TransCelerate Clinical Trial Diversification team.

What Was The Clinical Trial Diversification Workstream?

This workstream aimed to improve the ability of sponsor organizations to achieve trial populations that are representative of the target indication by increasing the racial and ethnic diversity of patients participating in clinical trials.

What Was The Vision?

Increased awareness of the need for representative clinical trials and adoption of practices aimed to improve the recruitment and retention of racially and ethnically diverse populations.

WHAT WAS THE APPROACH?

- *Identify practices and tools that sponsors could adopt and/or modify to improve minority recruitment and retention*
- *Develop guidance for each of the identified practices including the purpose of the practice and implementation strategies*
- *Disseminate and raise awareness of the identified practices, tools and guidelines to enhance the Sponsor's ability to achieve representative clinical trial diversity*
- *Primary focus on the U.S.*

Objectives and Target Audience

THE OBJECTIVES OF THE FOLLOWING CONTENT IS TO:

Provide sponsor companies with guidance on approaches for patient engagement and how they can successfully be used to enable recruitment and retention of minorities within clinical trial populations.

Content provided in this presentation is intended for sponsor company resources responsible for supporting trial execution and / or overseeing aspects of Clinical Operations relating to patients. In particular, those in the following roles:

Medical

Clinical Operations *(Protocol Managers & Site Managers)*

Site monitors

Regulatory Affairs

Any role which includes responsibility for patient engagement and diversity

May not necessarily be a formal, full time role

Executive Summary

Clinical trial populations are often not reflective of the target population who will use the medicine:

- Drug safety and efficacy can vary across demographic sub-groups
- Minorities have historically been under represented in clinical trial populations
- Addressing the lack of diversity in clinical trial populations is an on-going challenge for sponsors

The status quo is being challenged:

- The racial/ethnic sub-group population is growing and will soon no longer be a minority
- Marketing approvals are expected to become more stringent as regulatory authorities request more representative trial populations
- Patients, physicians and payers are increasingly demanding evidence of sub-group health outcomes
- The drive towards personalized medicine will likely require sponsors to be more expansive in their recruitment practices

KEY QUESTION:

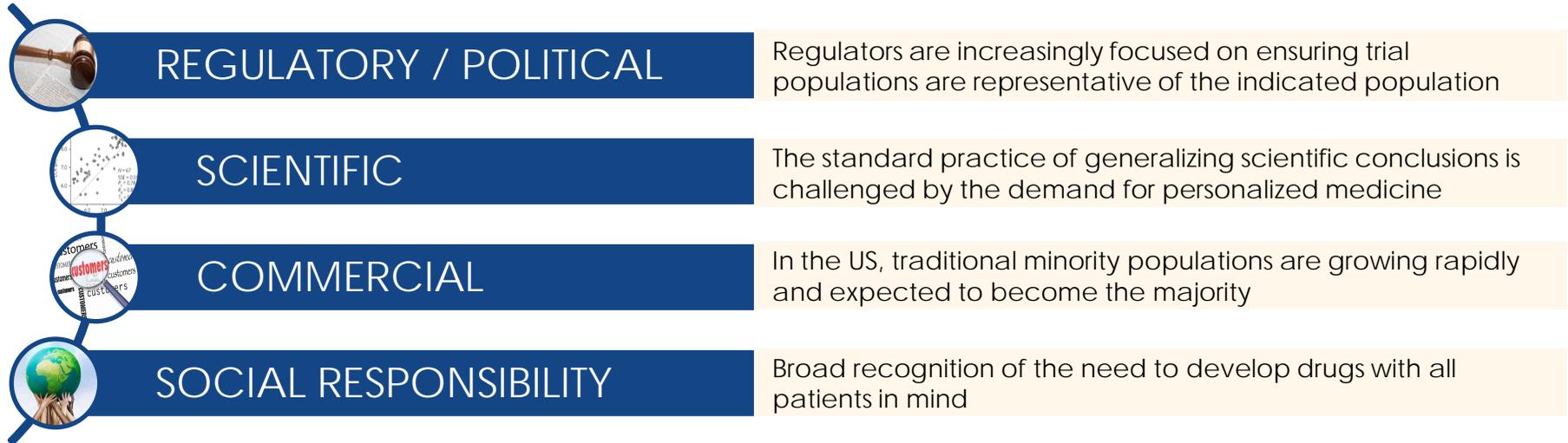
How can sponsors improve racial / ethnic diversity in clinical trial populations to better reflect the targeted population sub-groups?

Recommendation:

The Clinical Trial Diversification initiative recommend sponsors consider the use of patient engagement techniques to find opportunities to better align patient and sponsor goals and allow the exchange of actionable insights between sponsor and patient.

Sponsors face increasing pressure to ensure clinical trial populations reflect the disease state

Changing pressures to ensure representative diversity in clinical trial populations:



Current FDA guidance^[1] states:

"Although FDA has long requested race and ethnicity data on subjects in certain clinical trials, the Agency has not previously made explicit recommendations on the categories to use when collecting and reporting the data"

However, minority populations generally remain under-represented in clinical trial populations^[2]

African Americans represent 12% of the U.S. population but only 5% of clinical trial participants.

Hispanics represent 16% of the U.S. population but only 1% of clinical trial participants.

[1] FDA Guidance for Industry Collection of Race and Ethnicity Data in Clinical Trials (available at <http://www.fda.gov/downloads/RegulatoryInformation/Guidances/ucm126396.pdf>). Accessed Mar 3, 2015

[2] FDA Consumer updates: Clinical Trials Shed Light on Minority Health (Available at <http://www.fda.gov/ForConsumers/ConsumerUpdates/ucm349063.htm>). Accessed Feb 15, 2015

Patient engagement can help address underlying causes of inadequate trial diversity

ISSUES

- Lack of adequate trial diversity
- Patients are not consulted when trial teams are determining a trial's clinical plan
- Patients are not fully aware of clinical trials as potential treatment options

CONSEQUENCES

- Lack of full understanding of treatment safety and efficacy
- Lower recruitment and retention rates
- Outcomes that are meaningful to the patient are not captured
- Potentially fewer treatment options available to patients

CURRENT STATE

"AS IS"

- Patients are not providing input into trial design
- Trial teams don't see the human story of the disease
- Trials are designed that are impractical to patients
- Limited engagement of patients
- Trial outcomes are not communicated to patients

← addressing PATIENT ENGAGEMENT enabling →

Systematic approaches for engaging with patients, patient groups and providers in order to:

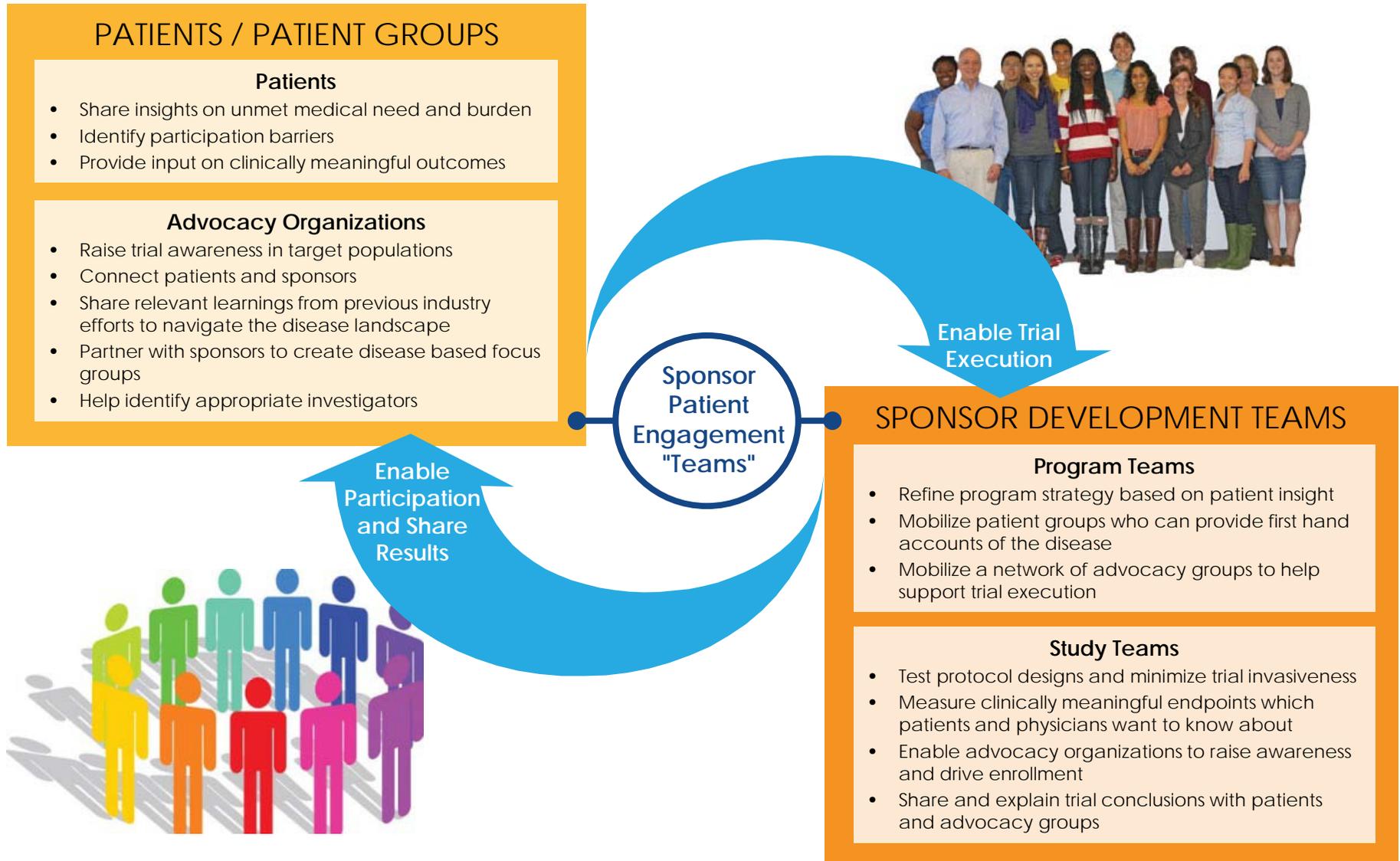
- Build disease intelligence of sponsors
- Better align trial and patient goals
- Identify investigators, SMEs and KOLs
- Identify patient participation barriers
- Match and navigate patients into clinical trials
- Educate patients

TARGET

"TO BE"

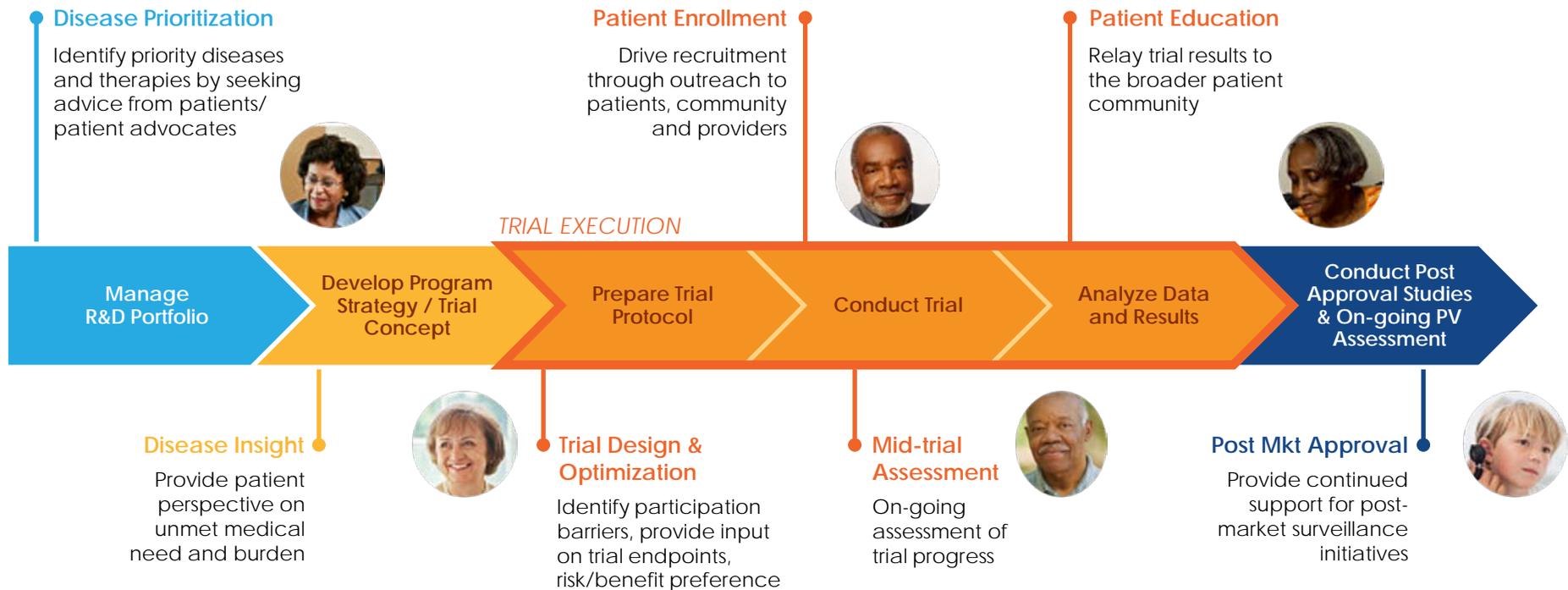
- Patients provide actionable insights throughout development
- Trials teams see the disease from the patient perspective
- Patient's provide feedback on trial invasiveness and practicality
- Trials are designed to be more appealing to the patient
- Trial findings are routinely communicated to patient groups

Patient engagement provides a platform for the mutually beneficial exchange of knowledge between sponsor and patient



The exchange of knowledge can provide actionable insights throughout the Clinical Trial process

There is no standard formula for using patient engagement techniques. However, there are key touch-points where key techniques may be applied.



Sponsors should consider a systematic approach for capturing the voice of the patient

When defining the strategic plan for patient engagement, ensure there is clarity within the trial team on your objectives, tactics and target outcomes.

THERAPEUTIC AREA / INDICATION PATIENT ENGAGEMENT STRATEGY & PLAN

What role can patient engagement play to support these objectives?

Designing Studies
Enrolling Patients
Raising Disease Awareness
Educating Patients
Advocating for Clinical / Research

- Define **objectives for patient engagement**
- Define the **scope and type** of patient engagement work
- **Partner** with Recruitment Manager, clinical teams and MDs – gain agreements
- Understand the current and future **patient profile of the disease**, where patients live and where investigators practice

Which organizations do we need to work with?

Advocacy Channel	National Influencers & Advocates			Regional/ State/ Local Influencers & Advocates		
	Tier 1 Groups (High Impact)	Tier 2 Groups (Advisory Focus)	Tier 3 Groups (Local Focus)	Tier 1 Groups (High Impact)	Tier 2 Groups (Advisory Focus)	Tier 3 Groups (Local Focus)
Public Education/ Disease Awareness		Partnership		Partnership		Partnership
Policy Advocacy	Partnership	Partnership	Partnership		Partnership	Partnership
Access Advocacy	Partnership	Partnership	Partnership		Partnership	Partnership
Clinical Research Advocacy						

- **Identify and prioritize** advocacy groups based on key criteria
- **Establish relationships** with advocacy groups (disease and community-based)

What activities will we deploy?

Type of Advocacy Work	Communication Channel	Objective of Change (Patient/Community Objective)	Specific Activity / Form of Collaboration or Support	SRM Resources Required
Patient Education/ Disease Awareness	Capability Assistance	Increase # patients treated - encourage drug uptake by key community health care providers across to non-urban	Engage with targeted community health centers on patient education via PCMEs Produce educational content materials for patients and medical organizations	Brand Advocacy PCMEs Support from USA
	Networking	Strengthen case system - Expand City 912 network within policy, advocacy, patient involvement funding	Provide access to drug networks Bi-annual meetings with City 912 leadership Share space with objectives Identify opportunities for visits City on	Brand Advocacy Support from USA
Policy Advocacy	Lobbying Support	Strengthen case system - Give City 912 access to drug independence operations on key issues of medical priorities	Provide access to drug networks Regular meetings with City 912 to ensure drug independence Assess City 912 priorities Have access to City 912 local patients to legislation which impacts funding	Brand Advocacy Support from USA
	Financial Support	Strengthen case system - Support City 912 efforts to expand case system to South by providing resources	Conduct training of operational initiatives Regular meetings with City 912 to understand funding process Engage with other areas - City is aligned	Brand Advocacy PCMEs

- Determine **key activities** to engage with patients or advocacy groups
- Capture best practices

How will we measure success?

Progress / Impact Measurements (Milestones)	
Critical Milestones	Status
	Red
	Yellow
	Green

- Identify **set of metrics and milestones** to track progress

Teams may need to be creative when defining the metrics for advocacy and patient engagement

The impact of Advocacy and patient engagement is difficult to quantify. Success is often measured by progress toward a particular goal.



Processes may need to be tailored in order to tie outcomes to specific techniques being employed.

Metrics need to be tailored to the techniques being used.

Examples metrics:

Outcomes related:

- Increased minority enrollment
- Reduced number of protocol amendments
- Enrollment timelines
- Improved quality of life for patients

randomizations that come from the portal /adverts

- Number of people in IVRS coming from advocacy
- Number of people referring to campaigns on social media (i.e. positive vs. negative comments)

Tactics related:

- Number of visits to a patient portal coming from advertizing
- Number of enrollments /

Execution related:

- Number of days delay for completion of planned activities

Organizations should also consider how to directly tie outcomes to direct financial benefits

(e.g. cost per protocol amendment x reduction in number of amendments)

Critical Success Factors When Building a Diversity Strategy for a Clinical Plan

- 
- ✓ Set company wide goals for representative trial populations
 - ✓ Understand the disease demographics, epidemiology and geography
 - ✓ Partner with patients and advocacy groups
 - ✓ Understand the patient needs and engage early
 - ✓ Gain buy in from key stakeholders

Additional Information

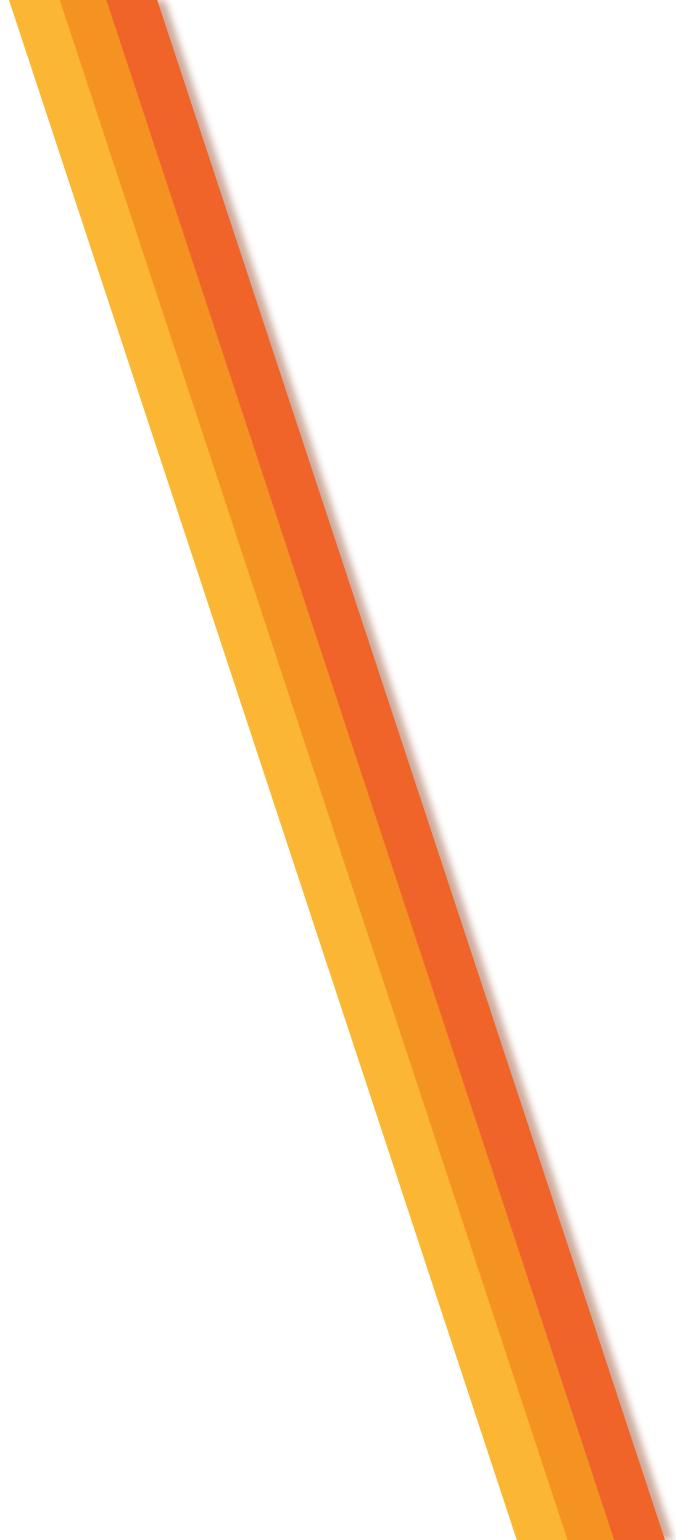
For any questions relating to this content,
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Further information can also be found
on the TransCelerate website

www.transceleratebiopharmainc.com

Appendices



Advocacy Organizations* (1/6)

Black / African American (1/2)

ORGANIZATION

WEBSITE

Below list provided by Karen Brooks, Pfizer (last updated 30-JAN-2015)

American Academy of Physical Therapy	www.aaptnet.org
Association of Black Cardiologists, Inc.	www.abcardio.org
Association of Black Psychologists	www.abpsi.org
Black Congress on Health, Law, and Economics (BCHLE)	www.bchle.org
The International Society on Hypertension in Blacks (ISHIB)	www.ishib.org
Minority Health Professions Foundation	www.minorityhealth.org
National Black Women's Health Project, Inc.	www.blackwomenshealth.org
National Dental Association	www.ndaonline.org
National Medical Association (NMA)	www.nmanet.org
National Optometric Association	www.natoptassoc.org
National Organization of Blacks in Dietetics and Nutrition (NOBIDAN)	www.nobidan.org
National Pharmaceutical Association	www.npha.net
Student National Medical Association (SNMA)	www.snma.org
American Black Chiropractic Association	www.abcachiro.com
Association of Black Women Physicians	www.blackwomenphysicians.org
Society of Black Academic Surgeons	www.sbas.net
National Black Nurses Association	www.npmaonline.org
National Podiatric Association	www.npmaonline.org
The Congress of National Black Churches	www.cnbc.org
Joint Center for Political and Economic Studies	www.jointcenter.org
National Association for the Advancement of Colored People (NAACP)	www.naacp.org

**Inclusion on this list does not indicate willingness to work with sites or sponsors*

Advocacy Organizations* (2/6)

Black / African American (2/2)

ORGANIZATION	WEBSITE
National Association for Equal Opportunity in Higher Education (NAFEO)	www.nafeo.org
National Association of Black Social Workers, Inc. (NABSW)	www.nabsw.org
National Caucus and Center on Black Aged, Inc. (NCBA)	www.ncba-aged.org
National Council of Negro Women (NCNW)	www.ncnw.org
National Urban League	www.nul.org
Sisters Network, Inc.	www.sistersnetworkinc.org
National Association of Black Journalists	www.nabj.org
National Council of Negro Women	www.ncnw.org
National Newspaper Publisher's Association	www.nnpa.org
100 Black Men of America	www.100blackmen.org/home.aspx
Blacks in Government	www.bignet.org
Sickle Cell Organization of the Inland Counties	
Black Biomedical Research Movement	www.bbrm.org
The Links Foundation	www.linksinc.org
The Coshar Foundation	www.cosharfoundation.org
National Association for Equal Opportunity in Higher Education (NAFEO)	www.nafeo.org
National Association of Black Social Workers, Inc. (NABSW)	www.nabsw.org
National Caucus and Center on Black Aged, Inc. (NCBA)	www.ncba-aged.org
National Council of Negro Women (NCNW)	www.ncnw.org
National Urban League	www.nul.org
Sisters Network, Inc.	www.sistersnetworkinc.org

*Inclusion on this list does not indicate willingness to work with sites or sponsors

Advocacy Organizations* (3/6)

Asian (1/2)

ORGANIZATION	WEBSITE
<i>Below list taken from National Partnership for Action (last updated 05-FEB-2015)</i>	http://minorityhealth.hhs.gov/npa/templates/content.aspx?lvl=3&lvlid=31&ID=274
ASPIRA Association, Inc.	http://www.aspira.org
Congressional Hispanic Caucus Institute (CHCI)	http://www.chci.org
Cuban American National Council, Inc.	http://www.cnc.org
Farmworker Justice	http://www.fwjjustice.org
Hispanic Association of Colleges and Universities	http://www.hacu.net
Hispanic Dental Association	http://www.hdassoc.org
Hispanic-Serving Health Professions Schools, Inc. (HSHPS)	http://www.hshps.org
Interamerican College of Physicians and Surgeons (ICPS)	http://www.icps.org
Inter-University Program for Latino Research (IUPLR)	http://www.nd.edu/~iuplr
League of United Latin American Citizens (LULAC)	http://www.lulac.org
MANA, A National Latina Organization	http://www.hermana.org
Migrant Legal Action Program	http://www.mlapp.org
National Alliance for Hispanic Health	http://www.hispanichealth.org
National Association of Hispanic Nurses	http://www.thehispanicnurses.org
National Association of Puerto Rican and Hispanic	http://www.naprhsw.com
National Council of La Raza (NCLR)	http://www.nclr.org
National Hispanic Council on Aging (NHCOA)	http://www.nhcoa.org
National Hispanic Medical Association (NHMA)	http://www.nhmamd.org
National Latina Health Organization	http://cnet.ucla.edu/women/nlho
National Latino Children's Institute (NLCI)	http://www.nlci.org
National Latino Council on Alcohol and Tobacco Prevention (LCAT)	http://www.nlcatp.org

*Inclusion on this list does not indicate willingness to work with sites or sponsors

Advocacy Organizations* (4/6)

Asian (2/2)

ORGANIZATION

WEBSITE

Below list taken from [Asian American Health](#) (last updated 05-FEB-2015)

<http://asianamericanhealth.nlm.nih.gov/national.html>

Association of Asian Pacific Community Health Organization

<http://www.aapcho.org/>

Asian and Pacific Islander American Health Forum

<http://www.apiahf.org/>

National Asian American Pacific Islander Mental Health Association

<http://naapimha.org/>

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Advocacy Organizations* (5/6)

American Indian Alaska Native

ORGANIZATION	WEBSITE
<i>Below list taken from National Tribal Organizations (last updated 04-FEB-2015)</i>	http://www.usa.gov/government/State-Local/Tribal-Sites/National-Tribal-Organizations.shtml
American Indian Heritage Foundation	http://www.indians.org/
American Indian Research and Policy Institute	http://www.airpi.org/
American Indian Ritual Object Repatriation Foundation	http://www.repatriationfoundation.org/index.html
International Indian Treaty Council	http://www.iitc.org/
National American Indian Housing Council	http://www.naihc.net/
National Center for American Indian Enterprise Development	http://ncaied.org/
National Congress of American Indians	http://www.ncai.org/
National Indian Gaming Association	http://www.indiangaming.org/
National Indian Gaming Commission	http://www.nigc.gov/
National Indian Health Board	http://www.nihb.org/
National Tribal Justice Resource Center	http://web.archive.org/web/20010515202310/http://www.tribalresourcecenter.org/
Native American Finance Officers Association	http://www.nafoa.org/
Native American Sports Council	http://www.nascsports.org/

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Advocacy Organizations* (6/6)

Hispanic / Latino

ORGANIZATION	WEBSITE
<i>Below list taken from National Partnership for Action (last updated 05-FEB-2015)</i>	http://minorityhealth.hhs.gov/npa/templates/content.aspx?vl=3&lvlid=31&iD=274
ASPIRA Association, Inc.	http://www.aspira.org
Congressional Hispanic Caucus Institute (CHCI)	http://www.chci.org
Cuban American National Council, Inc.	http://www.cnc.org
Farmworker Justice	http://www.fwjjustice.org
Hispanic Association of Colleges and Universities (HACU)	http://www.hacu.net
Hispanic Dental Association	http://www.hdassoc.org
Hispanic-Serving Health Professions Schools, Inc. (HSHPS)	http://www.hshps.org
Interamerican College of Physicians and Surgeons (ICPS)	http://www.icps.org
Inter-University Program for Latino Research (IUPLR)	http://www.nd.edu/~iuplr
League of United Latin American Citizens (LULAC)	http://www.lulac.org
MANA, A National Latina Organization	http://www.hermana.org
Migrant Legal Action Program	http://www.mlap.org
National Alliance for Hispanic Health	http://www.hispanichealth.org
National Association of Hispanic Nurses	http://www.thehispanicnurses.org
National Association of Puerto Rican and Hispanic Socia	http://www.naprhsw.com
National Council of La Raza (NCLR) Raul Yzaguirre Building	http://www.nclr.org
National Hispanic Council on Aging (NHCOA)	http://www.nhcoa.org
National Hispanic Medical Association (NHMA)	http://www.nhmamd.org
National Latina Health Organization	http://clnet.ucla.edu/women/nlho
National Latino Children's Institute (NLCI)	http://www.nlci.org
National Latino Council on Alcohol and Tobacco Prevention (LCAT)	http://www.nlcatp.org
Pan American Health Organization (PAHO)	http://www.paho.org
Society for Advancement of Chicanos and Native Americans in Science (SACNAS)	http://www.sacnas.org
U.S.- Mexico Border Health Commission	http://www.borderhealth.org/index.php

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