

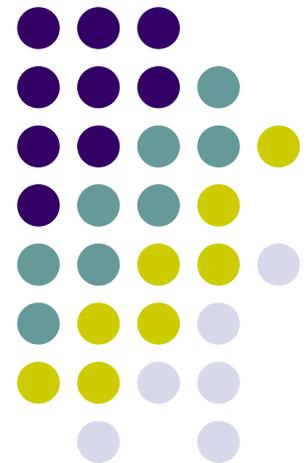
Peripheral Neuropathy Project Portfolio Analysis

Tina Tockarszewsky
The Neuropathy Association

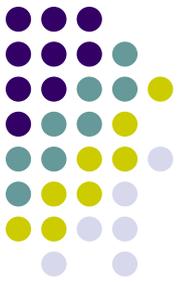
Linda Porter, Ph.D.

NIH/NINDS

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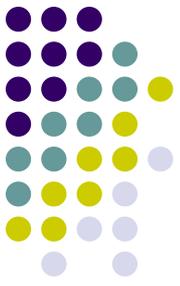


Peripheral Neuropathy (PN) Portfolio Overview



- As a grouped condition, PN included:
 - Painful diabetic peripheral neuropathy (DPN)
 - HIV/AIDS painful neuropathy
 - Charcot-Marie-Tooth (CMT) pain
 - Chemotherapy-induced painful neuropathy (CIPN)
 - Post-herpetic neuralgia (PHN)
 - Chronic inflammatory demyelinating polyneuropathy (CIDP) pain/painful autoimmune neuropathies
 - Carpal tunnel and other nerve entrapments
- Not included--although have painful PN involvement:
 - Celiac, Lyme, Idiopathic Neuropathy, Small Fiber Neuropathies

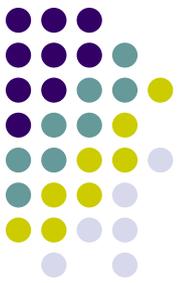
Prevalence of Peripheral Neuropathies



The millions in U.S. with peripheral neuropathy include:

- 15-18 million with diabetic peripheral neuropathy (*Neuropathy Association*);
- 79 million with pre-diabetes who are at risk for developing DPN (*CDC*);
- 230,000-575,000 with HIV-neuropathy, or 20-50% of HIV patients (*CDC*);
- 420,000 with chemotherapy-induced neuropathy, or 30% of cancer survivors (*American Cancer Society*);
- 125,000 with Charcot-Marie Tooth hereditary neuropathy (*CDC*).

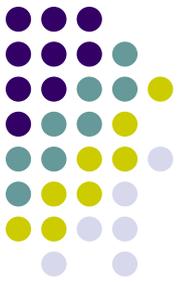
Peripheral Neuropathy Project Portfolio Overview



| | <u># of projects</u> | <u>% of projects</u> |
|----------------------|----------------------|----------------------|
| Basic | 39 | 65% |
| Translational | 18 | 30% |
| Clinical | 24 | 40% |

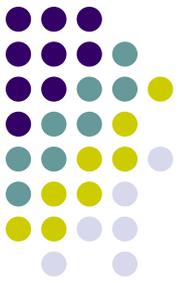
- **69 projects identified across the pain portfolio.**
- Projects found across NIH institutes and at the VA--but at no other agencies.
- Basic science constituted core of the portfolio.
- Less than a third of the projects had a translational research component.
- Less than 50% had a clinical research component.
- Of the 39 projects with a basic science component, 28 projects were 100% basic science (40% of PN portfolio).
- Of the 24 projects with a clinical research component, 16 projects were 100% clinical research (23% of PN portfolio)—majority of these projects address diabetes or PHN, with only one project each for CIPN and HIV neuropathy.

Top Tier 2 Categories in the Peripheral Neuropathy Project Portfolio



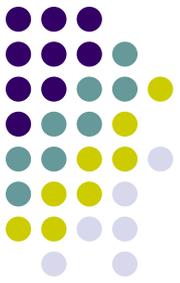
| | |
|--|-----|
| •Neurobiological/glia mechanisms of nociception and pain | 26% |
| •Mechanisms of and treatments for transitions in pain phase | 10% |
| •Development and validation of animal and human pain models | 8% |
| •Genetics and genomics of nociception and pain | 7% |
| •Pharmacological mechanisms and treatment | 7% |
| •Training in pain research | 7% |
| •Analgesic development | 6% |
| •Non-pharmacological mechanisms and treatment | 5% |
| •Unique populations (<i>only pediatric-CMT and elderly considered</i>) | 5% |
| •Biobehavioral and psychosocial mechanisms and treatment of pain | 3% |
| •Development of device and therapy delivery systems | 3% |
| •Pain prevention | 3% |
| •Pain outcomes assessments/measures; novel health IT as tools for decision making support of pain management | 2% |

Lowest Tier 2 Categories in the Peripheral Neuropathy Project Portfolio



| | |
|---|----|
| • Other “omics” of pain: | 1% |
| • Diagnosis/case definitions | 1% |
| • Medical management of pain | 1% |
| • Health disparities in pain, pain management, and access to care | 1% |
| • Pain and women’s and minority’s health research | 1% |
| • Pain and trauma | 1% |
| • Chronic overlapping pain conditions in an individual | 1% |
| • Pain and other non-pain co-morbidities | 1% |
| • Development of informatics, data bases, and IT as tools for pain research | 0% |
| • Pain education | 0% |
| • Epidemiology of pain and pain disorders | 0% |
| • Sex and gender differences in pain | 0% |
| • Comparative effectiveness research | 0% |
| • Pain and substance use and abuse/addiction | 0% |
| • Analgesic drug safety | 0% |
| • Health care utilization | 0% |

Notable in Portfolio Review



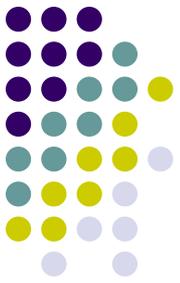
- Emphasis on basis science not surprising given need for more knowledge to lead to clinical development of disease-modifying agents for PN
 - Greater focus needed on nerve repair, nerve regeneration, neuroprotective models
 - Work on HIV-PN neuroprotective models, DPN autonomic neuropathy human models to prevent amputations hold great promise
- PHN only getting attention for prevention
- Research training only focused on DPN
- Only unique populations addressed are pediatric and elderly



Notable in Portfolio Review (cont.)

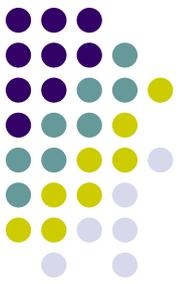
- With emphasis on basic research, patients left in care and treatment black hole:
 - Challenges in accessing existing care, treatment, supportive therapeutic options
 - Comparative effectiveness of available therapies not addressed
 - Biopsychosocial aspects non-existent
 - Epidemiology does not exist
 - Co-morbidities and overlapping conditions not being considered
 - Lack of education presents barriers to care

Relevance to Other Pain Conditions/Collaboration Opportunities



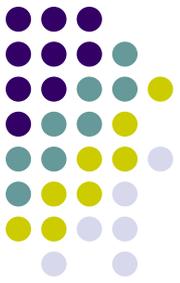
- Broad base of untapped opportunities both to generate basic science research to benefit many as well as leverage existing care models on behalf of neuropathic pain communities
- Neuroprotective mechanisms could be relevant to all toxic neuropathies, provide support against viral/biochemical imbalances
- Nerve repair/regeneration research: direct correlation to all neuropathic pain conditions
- Shared relevance for education and healthcare utilization for other neuropathic pain conditions.

Potential Overlap/Shared Interests Among Agencies or NIH Institutes



- Given its “footprint,” PN should have been found across the entire government research portfolio
- PN/neuropathic pain ripe for platform/roadmap that is cross-agency/cross-institute
- Current research across the portfolio does not neatly connect to effect real change
- Pain component across the spectrum of neuropathies not adequately realized and recognized by researchers and clinicians alike

Research Gaps and Opportunities



- Greater emphasis needed on nerve repair, nerve regeneration, neuroprotective mechanisms
- Greater emphasis on the areas that are impacting patients NOW: education, access to all therapies available, integrative approaches, better understanding of co-morbidities and overlapping conditions so care moves into the realm of “practical”
- Epidemiological studies would help strengthen documentation of prevalence and healthcare trends to protect access to care until better therapies and cures are found.