Drug Discovery for Neglected Tropical Diseases
NTDs: What Are They?

• Diverse group of infectious diseases
• Many are vector-borne
• Risk factors:
  • Poverty
  • Poor sanitation
  • Close contact with vectors
  • Underdeveloped medical systems
NTDs: Where Are They?

NTDs: What Is Their Impact?

- 11% of global disease burden
- 3 Billion people at risk
- 1.6 Billion people have at least one NTD
- 500,000 will die each year
- Patients face many challenges:
  - Pain and suffering
  - Stigmatization
  - Obstacles to growth

River Blindness (Onchocerciasis) patient, Cameroon

NTDs: How Are We Fighting Them?

- Preventative chemotherapy
- Innovative disease management
- Vector control
- Safe drinking water, sanitation, hygiene, education
- Veterinary science
- Government and philanthropy

NTDs: Have We Been Successful?

• Only 2% of medical R&D funds
• 1% of approved NCEs
• Three Highlights:
  • New cases of Guinea-worm disease down 99% since 1989
  • 853 Million people treated with preventative chemotherapy in 2014
  • 77% decrease in reported HAT cases since 1999
Drug Discovery: Aspirin

• Use chemical compound to modulate biological effect
  • reduce pain, inflammation

• Binds to an enzyme
  • Cyclooxygenase

• Originally derived from nature
  • Bark of the willow tree

• Has been used for at least 2400 years

Willow tree

http://img.wonderhowto.com/img/77/01/63496526784953/0/make-aspirin-from-willow-tree.w654.jpg
Cost of Drug Discovery

• $2.6 Billion per approved compound
  • $1.4 Billion out-of-pocket cost
  • $1.2 Billion time costs
• Post-approval R&D adds even more cost
  • $312 Million per drug
• Based on 106 drugs tested between 1995 and 2007
  • Global, not just U.S.
• Obviously a huge hurdle for NTD drug discovery

http://csdd.tufts.edu/news/complete_story/pr_tufts_csdd_2014_cost_study
FDA Priority Review Vouchers

• First proposed in 2006
• Encourages companies to develop drugs for NTDs
• Rewards them with expedited review
• Vouchers can be used at any time or sold
• Very popular
• Has lead to 3 NTD drugs and 6 rare pediatric disease drugs

Challenges to Drug Discovery for NTDs

• What do you think makes NTD drug discovery especially difficult?

• Do you think the same regulatory process should be used for NTDs?